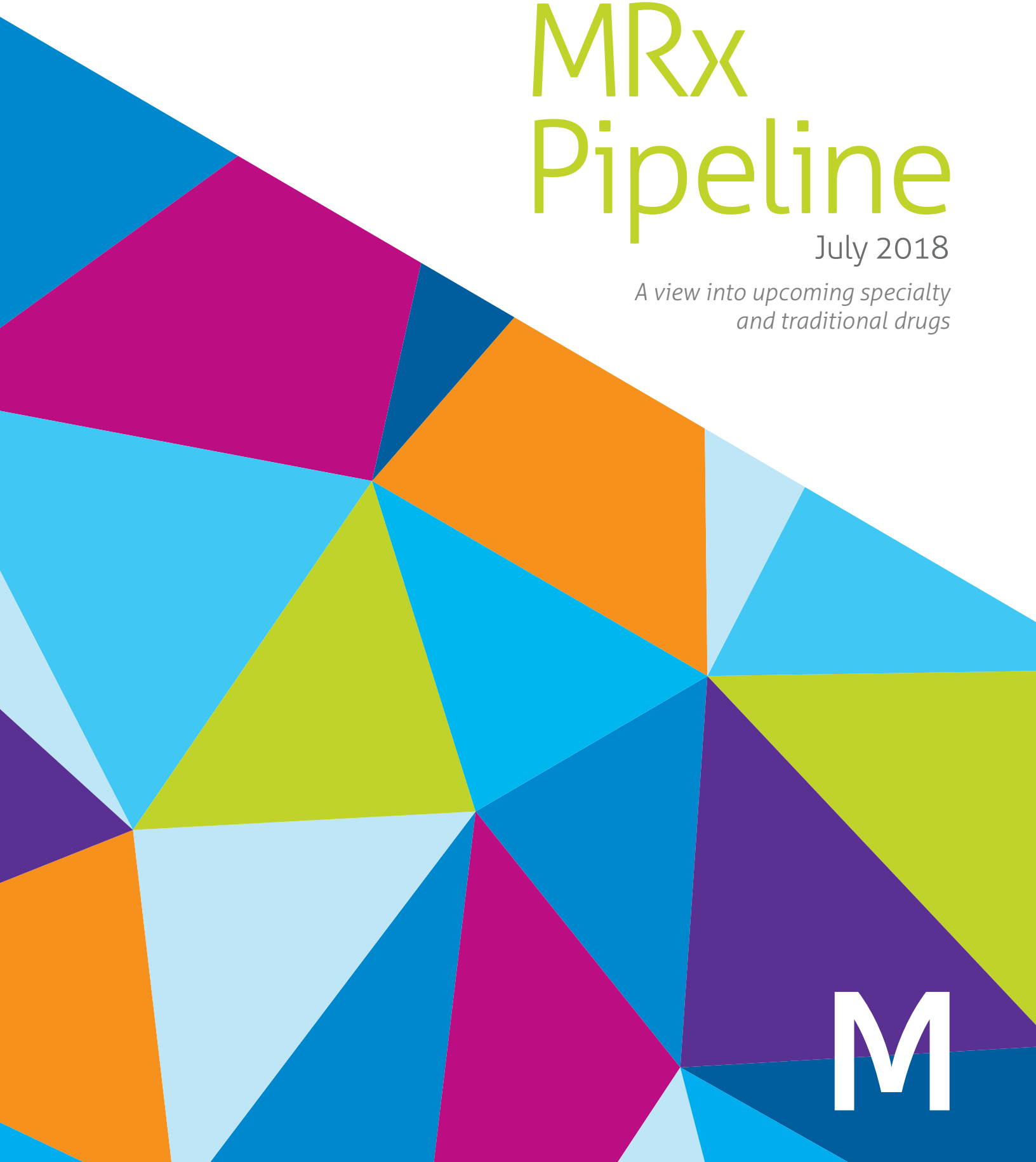
Magellan Rx MANAGEMENT℠

# MRx Pipeline

July 2018

*A view into upcoming specialty and traditional drugs*



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# INTRODUCTION

Welcome to the *MRx Pipeline*. In its second year of publication, this quarterly report offers clinical insights and competitive intelligence on anticipated drugs in development. Our universal forecast addresses trends applicable across market segments.

Traditional and specialty drugs, agents under the pharmacy and medical benefits, new molecular entities, pertinent new and expanded indications for existing medications, and biosimilars are profiled in the report.

Clinical analyses, financial outlook, and pre-regulatory status are considered as part of the evaluation process. The products housed in the *MRx Pipeline* have been researched in detail and developed in collaboration and in consultation with our internal team of clinical and analytics experts.

Emerging therapeutics continue to grow and influence the clinical and financial landscape. Therefore, Magellan Rx Management has developed a systematic approach to determine the products with significant clinical impact. For the in-depth clinical evaluations, the products' potential to meet an underserved need in the market by becoming the new standard of care and the ability to replace existing therapies were investigated. The extent to which the pipeline drugs could shift market share on a formulary and their impact on disease prevalence were also important considerations.

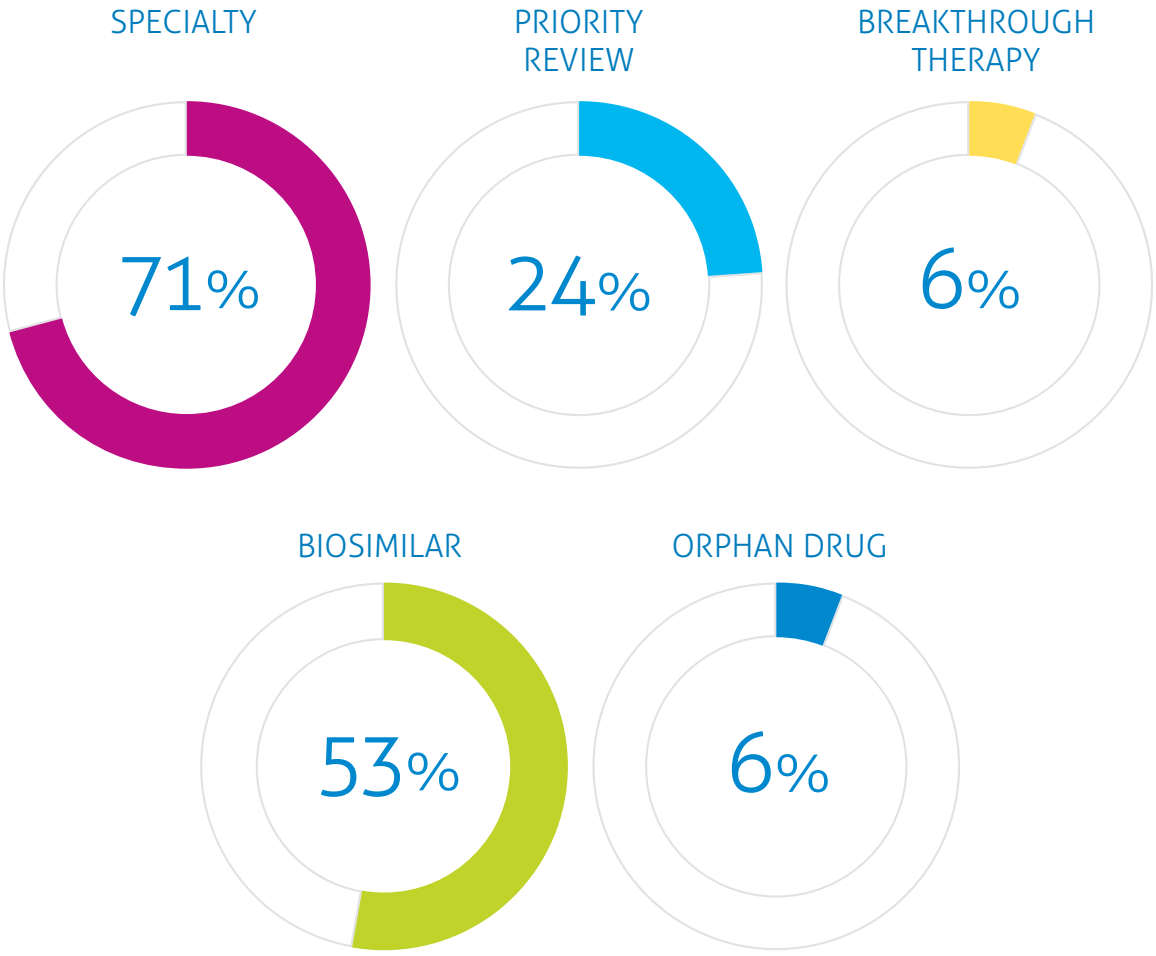
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year 2022. These figures are not specific to a particular commercial or government line of business; rather, they look at forecasted total US sales. Depending on a variety of factors, such as the therapeutic category, eventual approved FDA indications, population within the plan, and other indices, the financial impact could vary by different lines of business.

In the past few years, game changers such as products in the hepatitis C field and chimeric antigen receptor (CAR)-T therapies have revolutionized standard of care. As we look ahead, a continued trend toward the approval of specialty medications, as well as the growth of biosimilars, digital therapeutics, and new treatment modalities using gene therapy are expected. Noteworthy pipeline trends to watch in the upcoming quarters include the development of complex therapies, therapeutic options for rare hereditary diseases, oncology, immunology, migraine prophylaxis, and Parkinson's disease. Moreover, investigational products for Alzheimer's disease, hemophilia, multiple sclerosis, women's health, and infectious diseases including influenza, await over the horizon.

The drug pipeline ecosphere will continue to evolve as it faces challenges and successes. Novel agents that apply innovation to show positive results, without compromising patient safety and access, offer true therapeutic advances and hold the promise to alter the treatment paradigm.

# Pipeline Deep Dive

Objective evidence-based methodology was used to identify the Deep Dive drugs in the upcoming quarters. This section features a clinical overview and explores the potential place in therapy for these agents. Moreover, it addresses their FDA approval timeline and 5-year financial forecast.




★ Specialty drug names appear in magenta throughout the publication.

Infectious Disease

# baloxavir marboxil *oral*

Genentech

## PROPOSED INDICATIONS

Treatment of uncomplicated influenza in patients ≥ 12 years of age

## CLINICAL OVERVIEW

Baloxavir inhibits cap-dependent endonuclease, an enzyme required for viral replication.

The phase 3 CAPSTONE-1 study performed in the US and Japan evaluated safety and efficacy of baloxavir in 1,436 otherwise healthy individuals, aged 12 to 64 years, with confirmed influenza virus infection. Baloxavir significantly improved the median time to alleviation of symptoms (TTAS), the primary endpoint, compared to placebo (53.7 versus 80.2 hours, respectively). A significantly faster resolution of fever was also reported with baloxavir (median, 24.5 versus 42 hours, respectively). TTAS and time to fever reduction were similar between baloxavir and oseltamivir; however, the proportion of patients with positive titers for influenza virus at 1, 2, and 4 days after the start of treatment was significantly lower for baloxavir compared to oseltamivir, as was the time to end of viral shedding (24 versus 72 hours, respectively; 96 hours with placebo). Baloxavir was well tolerated. The most common adverse effects were diarrhea, bronchitis, nausea, and sinusitis, all of which occurred less often with baloxavir than with placebo. The phase 3 CAPSTONE-2 trial in patients ≥ 12 years old with a high risk of influenza is ongoing. Baloxavir has not been studied in hospitalized patients or in patients < 12 years of age.

Baloxavir was evaluated as single oral doses of 1) 40 mg in patients weighing < 80 kg; and 2) 80 mg in adult and adolescent patients weighing ≥ 80 kg. In comparison, oseltamivir was given as 75 mg twice daily for 5 days in adults only. Study drugs were administered within 48 hours of symptom onset.

## PLACE IN THERAPY

Influenza places a considerable burden on the US healthcare system each year. The estimated annual incidence of events due to the flu in the US include 9.2 to 35.6 million influenza cases, 140,000 to 710,000 hospitalizations, and 12,000 to 16,000 deaths. The 2017-2018 flu season was particularly severe in the US, with influenza A(H3N2) predominating throughout the majority of the season.

While yearly vaccination to prevent the flu is the main course of attack, treatment with antiviral agents can lessen symptoms, shorten duration of illness, and prevent serious complications. Currently recommended antivirals to treat and/or prevent influenza after exposure include oral oseltamivir (Tamiflu® and generics), inhaled zanamivir (Relenza®), and IV peramivir (Rapivab™; treatment only). All 3 agents are classified as neuraminidase inhibitors (NAI).

Yearly variations of vaccine effectiveness, combined with emerging drug resistance, require the development of new drugs to treat influenza infections. New agents that disrupt RNA polymerase activity, important for viral replication, are on the horizon and may have broad efficacy against multiple virus strains and subtypes. Baloxavir, which was approved in Japan in February 2018, is a first-in-class influenza antiviral with a convenient single-dose oral regimen. It provides a unique mechanism of action against influenza A and B viruses, including strains that are resistant to oseltamivir and avian strains (H7N9, H5N1). Other oral agents that target RNA polymerase in phase 3 trials include favipiravir (Medivector), a 2-dose regimen to treat influenza A, B, and C in adults that is already approved in Japan, and pimodivir (Janssen), a 5-day regimen for influenza A in adults and adolescents, including hospitalized or at-risk patients.

## FDA APPROVAL TIMELINE

December 24, 2018

✓ Priority review

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $105 | $150 | $192 | $229 | $262 |

The forecast is a projection of total US sales per year.

Oncology

# cemiplimab *IV*

Regeneron

## PROPOSED INDICATIONS

Treatment of metastatic cutaneous squamous cell carcinoma (CSCC) or locally advanced CSCC in patients who are not candidates for surgery

## CLINICAL OVERVIEW

Cemiplimab is a programmed cell death protein-1 (PD-1) checkpoint inhibitor.

An open-label, phase 2 trial evaluated cemiplimab in 59 patients with metastatic CSCC. After a median follow-up of 7.9 months, an overall response rate (ORR) of 47.5% was reported (complete response [CR] rate was 6.8% and partial response [PR] rate was 40.7%). Median time to response was 1.9 months and durable disease control rate (DCR), defined as proportion of patients without progression for ≥ 105 days, was 61%. The median duration of response and median progression-free survival (PFS) were not reached at data cutoff. Of the patients who responded, 82% remained in response and continued treatment. Responses were seen in patients with or without prior systemic therapy. Diarrhea, fatigue, and nausea were the most commonly reported adverse effects. The rate of grade 3 or greater events was 42%. Three deaths due to adverse events and 8 deaths due to disease progression were reported.

The phase 2 study in patients with locally advanced CSCC is ongoing. The time point of the primary analysis has not been reached; however, of 10 patients in the phase 1 expansion cohort with locally advanced disease who were included in the phase 2 study, 6 patients had an objective response.

Cemiplimab was studied at doses of 3 mg/kg infused IV over 30 minutes every 2 weeks for 96 weeks or until disease progression.

## PLACE IN THERAPY

CSCC is the second most common skin cancer in the US, with over 700,000 new cases diagnosed each year. While risk of metastasis is low (5%), CSCC can result in substantial disfigurement involving soft tissue, cartilage, and bone. Surgical approaches or radiation therapy are typical modalities to treat localized CSCC, with reported cure rates of 95%. The choice of therapy depends on functional considerations, cosmetic outcomes, and patient preference. Regional involvement of lymph nodes significantly increases the risk of recurrence and mortality. No approved systemic therapy exists for metastatic CSCC or advanced CSCC with local involvement that is inappropriate or unresponsive to surgery or radiation therapy. While other agents that inhibit PD-1 are approved to treat melanoma, if approved, cemiplimab will be the first systemic agent to treat advanced CSCC. Cemiplimab is also in late stage trials for cervical cancer and NSCLC. Another, PD-1 inhibitor, pembrolizumab (Keytruda®), is in phase 2 trials for recurrent or metastatic CSCC.

## FDA APPROVAL TIMELINE

October 26, 2018

✓ Breakthrough therapy ✓ Priority review

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $22 | $94 | $191 | $214 | $223 |

The forecast is a projection of total US sales per year.

Oncology

# dacomitinib *oral*

Pfizer

## PROPOSED INDICATIONS

First-line treatment of locally advanced or metastatic non-small cell lung cancer (NSCLC) in patients with epidermal growth factor receptor (EGFR)-activating mutations

## CLINICAL OVERVIEW

Dacomitinib is a pan-human EGFR tyrosine kinase inhibitor (TKI). It blocks the signaling in both wild-type and EGFR-mutated NSCLC, including forms resistant to human epidermal growth factor receptor (HER) inhibitors.

Submission of dacomitinib is supported by the open-label ARCHER 1050 trial that randomly compared dacomitinib with gefitinib in 452 newly diagnosed patients with advanced or recurrent NSCLC with EGFR-activating mutation (exon 19 deletion or exon 21 L858R mutation ± exon 20 T790M mutation) and without CNS involvement. Median PFS was 14.7 months with dacomitinib and 9.2 months with gefitinib (HR 0.59). There was a significant improvement in overall survival (OS) with dacomitinib compared to gefitinib (median 34.1 versus 26.8 months; HR 0.76). At 30 months, survival rates were higher with dacomitinib (56.2% and 46.3%, respectively). Common adverse effects reported with dacomitinib were diarrhea (including grade 3-5), nail changes, rash/dermatitis acneiform (including grade 3), and mouth sores. Discontinuation rates due to side effects were 10% for dacomitinib and 7% with gefitinib.

Dacomitinib was studied in doses of 45 mg daily in 28-day cycles until disease progression or other discontinuation criteria was met.

## PLACE IN THERAPY

Lung cancer is the leading cause of cancer death in the US. It is estimated in 2018 that over 234,000 new cases and over 150,000 related deaths will occur. While the 5-year survival rate of lung cancers overall is 18%, notable progress has been made to target therapies for patients with specific gene mutations.

NSCLC accounts for 80% to 85% of all lung cancers and most patients present with advanced or metastatic disease upon diagnosis. EGFR mutations are more common in non-smokers, women, and Asians. The most common EGFR mutations are exon 19 deletion and exon 21 L858R, which are sensitive to EGFR TKIs (e.g., erlotinib [Tarceva®], afatinib [Gilotrif®], gefitinib [Iressa®], or osimertinib [Tagrisso®]), making these products first-line treatments in patients with EGFR mutations. The T790M mutation has been linked to acquired resistance to EGFR TKI therapy and is found in about 60% of patients with failure to these agents.

While the first-generation EGFR TKIs, gefitinib and erlotinib, reversibly inhibit EGFR-mutated NSCLC, the second-generation agents, afatinib and dacomitinib, cause irreversible inhibition to EGFR and also to other HER receptors. The third generation osimertinib is also an irreversible EGFR TKI, with activity against T790M-resistant mutations. Dacomitinib is the first EGFR TKI to show a significant clinically meaningful improvement in PFS over gefitinib, but it is met with greater toxicity and need for dose reduction. Further head-to-head trials, particularly against osimertinib in patients with T790M mutations and CNS metastasis, are needed to determine if dacomitinib will replace existing EGFR TKIs in this setting.

## FDA APPROVAL TIMELINE

September 2018

✓ Orphan drug ✓ Priority review

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $8.3 | $47 | $71 | $102 | $137 |

The forecast is a projection of total US sales per year.

## BACKGROUND

It is estimated that up to 1 million Americans are living with Parkinson's disease (PD) and over 60,000 new cases are diagnosed each year. Carbidopa/levodopa (CD/LD) is a mainstay in the treatment of PD. Over time, unfortunately, its use is limited by "off" periods when the medication stops working suddenly or at the end of a dosing interval, at which time patients experience motor fluctuations. Adjusting the dose and dosing frequency is the first step in managing "off" episodes. Switching to an ER formulation has not consistently demonstrated benefit. CD/LD is also available as an intestinal infusion pump (Duopa™), administered through a percutaneous gastrojejunostomy tube to provide 16 hours of continuous treatment. Other approaches include addition of a dopamine agonist, catechol-O-methyl transferase (COMT) inhibitor, or monoamine oxidase (MAO) B inhibitor. Apomorphine (Apokyn®) is available via a SC auto-injector for rescue treatment of sudden and severe "off" episodes. It has an onset of action of about 10 minutes and necessitates premedication with an antiemetic.

# apomorphine (APL-130277) *SL*

Sunovion

## PROPOSED INDICATIONS

Treatment of motor fluctuations ("off" periods) in patients with PD treated with CD/LD

## CLINICAL OVERVIEW

Apomorphine is a fast-acting, non-ergoline dopamine agonist formulated as a SL film.

A phase 3 trial evaluated apomorphine SL film in 219 patients with PD who were on stable doses of CD/LD and experienced early morning "off" episodes. At 12 weeks, the study reported significant improvement of motor function with apomorphine SL as demonstrated by a difference in the Movement Disorder Society UPDRS (MDS-UPDRS) score of 7.6 points 30 minutes after dosing compared with placebo. Statistical significance was seen as early as 15 minutes post-dose and lasted up to the last measured time point at 90 minutes. Similar results were seen at 4 and 8 weeks. The study reported that a significantly higher percentage of patients treated with apomorphine SL achieved a full "on" response within 30 minutes of administration compared to placebo. The most frequent adverse effects were nausea (28%), somnolence (13%), and dizziness (9%).

Patients were titrated to an effective dose of apomorphine SL ranging from 10 mg to 35 mg administered up to 5 times per day.

## FDA APPROVAL TIMELINE

January 29, 2019

*Apomorphine SL was submitted to the FDA via the abbreviated 505(b)(2) pathway pathway, in which at least some of the information required for approval is obtained from studies not conducted by or for the applicant.*

✓ Fast track

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $8 | $32 | $60 | $92 | $120 |

The forecast is a projection of total US sales per year

# levodopa (Inbrija) *inhalation*

Acorda

## PROPOSED INDICATIONS

Treatment of symptoms associated with CD/LD "off" periods in patients with PD

The 12-week, phase 3 SPAN-PD trial evaluated levodopa inhalation as rescue treatment for motor fluctuations in 339 patients with PD who require a levodopa-containing medication dosed at least 4 times daily and experience ≥ 2 hours mean time of motor fluctuations per day. Levodopa inhalation doses of 84 mg, taken up to 5 times per day, led to a significant improvement in motor function 30 minutes post-dose compared to placebo as measured by the mean change in Unified PD Rating Scale – Part 3 (UPDRS3; -9.8 versus -5.9 points); the effect persisted through 60 minutes post-dose. Nominal improvement was seen at 10 minutes post-dose, with a trend toward improvement at 20 minutes. The study also demonstrated statistically significant improvement 30 and 60 minutes after a dose of 60 mg, a secondary endpoint. Based on the Patient Global Impression of Change (PGI-C), improvement was dose dependent, and over twice as many patients treated with levodopa 84 mg reported improved or much-improved symptoms compared to placebo-treated patients. The most common adverse effects reported were cough (15%), upper respiratory tract infection (6%), nausea (5%), and discolored sputum (5%). Overall, 85% of patients who received levodopa and 87% of those on placebo completed the study. Additional studies showed no impact on lung function in patients without chronic lung disease or asthma. Reversible, asymptomatic, acute bronchoconstriction was reported in a small group of patients (10/25) with mild or moderate asthma. One 52-week study assessing levodopa inhalation (mean, 2.3 doses of 84 mg/day) reported improvement based on PGI-C in 73% of patients and a reduction in "off" time of 1.15 hours.

## FDA APPROVAL TIMELINE

October 5, 2018

*Levodopa inhalation was submitted to the FDA via the abbreviated 505(b)(2) pathway, in which at least some of the information required for approval is obtained from studies not conducted by or for the applicant.*

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $1 | $81 | $185 | $276 | $371 |

The forecast is a projection of total US sales per year.

## PLACE IN THERAPY

"Off" episodes are experienced by 40% to 60% of patients with PD and may worsen with disease progression. "Off"-episodes generally occur in the morning after awakening and periodically throughout the day. These episodes impact a person's ability to perform activities of daily living. Dose modification and medication supplementation may reduce "off"-episodes, but these strategies may not be as helpful in advanced PD.

If approved, SL apomorphine and inhaled levodopa may provide fast-acting rescue treatment options for motor fluctuations experienced during "off" episodes. Both offer a more convenient and patient-friendly approach than SC injected apomorphine (Apokyn), the only FDA-approved product for rescue therapy. Uptake of either product will depend on patient acceptance and QOL impact.

Respiratory

# mepolizumab (Nucala®) *SC*

GlaxoSmithKline

## PROPOSED INDICATIONS

Chronic obstructive pulmonary disease (COPD) with an eosinophilic phenotype as add-on to maintenance treatment

*Mepolizumab is currently FDA-approved as add-on maintenance treatment of severe asthma in patients aged ≥ 12 years with an eosinophilic phenotype and for the treatment of adults with eosinophilic granulomatosis with polyangiitis (EGPA).*

## CLINICAL OVERVIEW

As with asthma, eosinophilic inflammation may be present in some patients with COPD. Mepolizumab inhibits interleukin-5 (IL-5), a major cytokine responsible for the maturation, recruitment, activation, and survival of eosinophils.

The 52-week METREX and METREO studies evaluated mepolizumab in patients with COPD and ≥ 2 moderate COPD exacerbations in the prior 12 months while on optimal standard background therapy, including inhaled corticosteroids (ICS). Treatment with mepolizumab 150 mg compared to placebo resulted in a reduction in moderate and severe exacerbations by 18% (METREX, significant change) and 20% (METREO, nonsignificant change) in patients with a blood eosinophil count > 150 cells/µL at study entry or > 300 cells/µL within the year prior to enrollment. Benefit of mepolizumab was not demonstrated in patients with baseline eosinophils < 150 cells/µL. No new safety concerns were identified that differed from the established indications.

Mepolizumab was studied at dosages of 100 mg and 300 mg SC every 4 weeks for 52 weeks. No benefit was reported with the 300 mg dose.

## PLACE IN THERAPY

COPD affects approximately 32 million Americans and is the third leading cause of death in the US. Up to 40% of patients with COPD may have eosinophilic phenotype (defined as blood eosinophil count ≥ 2% [≥ 150 to 200 cells/µL]). Some studies suggest that blood eosinophils may be a biomarker of COPD exacerbation risk and may predict response to ICS therapy.

Current guidelines recommend triple-therapy with a combination of an ICS, a long-acting beta-agonist, and a long-acting muscarinic antagonist in patients who experience frequent exacerbations. If exacerbations still occur with triple-therapy, then the oral phosphodiesterase 4 (PDE4) inhibitor, roflumilast (Daliresp®), may be added.

While mepolizumab's use for COPD has been met with mixed results, if approved, it will be a first-in-class agent for this indication. Pulmonologists and allergists may prescribe mepolizumab in a limited subset of patients with severe COPD, characterized by frequent exacerbations and high eosinophil levels, who have no other options.

## FDA APPROVAL TIMELINE

September 7, 2018

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $428 | $507 | $561 | $617 | $667 |

The forecast is a projection of total US sales per year for bundled indications.

# omadacycline *IV, oral*

Paratek

## PROPOSED INDICATIONS

Acute bacterial skin and skin-structure infections (ABSSSI) and community-acquired bacterial pneumonia (CABP)

## CLINICAL OVERVIEW

Omadacycline is a tetracycline-derived aminomethylcycline antibiotic with activity against gram-positive, gram-negative, atypical, and anaerobic bacteria, including bacteria that are resistant to other agents.

OASIS-1 (n=645; IV to oral) and OASIS-2 (n=735; oral only) studies compared once-daily omadacycline and twice-daily linezolid in patients with methicillin- or multidrug-resistant *Staphylococcus aureus* (MRSA, MDR) ABSSSI. At baseline, 61% of *S. aureus* isolates were resistant to ≥ 1 antibiotic class. Pooled data demonstrated overall high clinical success rates of 83% for omadacycline and 81% for linezolid. Common adverse effects included GI symptoms (e.g., nausea, vomiting, diarrhea), which occurred at similar rates with both agents.

Once-daily IV-to-oral regimens of omadacycline and moxifloxacin were compared in the phase 3 OPTIC trial in 774 adults with CABP, including cases with MRSA. Pneumonia severity index (PORT scores) ranged from class II to IV. Clinically stable patients were transitioned to oral therapy after a minimum of 3 days of IV administration. In the intent-to-treat group, the Early Clinical Response (ECR) rate, assessed 3 to 5 days after the first dose, was 81% with omadacycline compared to nearly 83% with moxifloxacin.

In OASIS-1 and OPTIC, omadacycline was given as 100 mg IV every 12 hours for 2 doses, followed by 100 mg IV every 24 hours for at least 3 days, followed by 300 mg orally every 24 hours.

## PLACE IN THERAPY

Over 2 million cases of skin and soft tissue infections were identified in the US from 2005 to 2010, with the vast majority treated in ambulatory settings. Mild cases can be treated with an oral penicillin, cephalosporin, or clindamycin. For empiric coverage of community-acquired MRSA, oral options include clindamycin, doxycycline, minocycline, linezolid (Zyvox®), and trimethoprim-sulfamethoxazole. For complicated MRSA cases, IV formulations of vancomycin, linezolid (IV or oral), daptomycin (Cubicin®), telavancin (Vibativ®), or clindamycin (IV or oral) are preferred. Newer agents, tedizolid (Sivextro®) and delafloxacin (Baxdela™), available as IV and oral dosages, and IV tigecycline (Tygacil®) are also indicated for ABSSSI; currently, their use is not addressed in practice guidelines.

In 2015, over 51,000 pneumonia-related deaths occurred in the US. Treatment with a macrolide (azithromycin, clarithromycin, erythromycin) is strongly recommended for outpatient treatment in otherwise healthy adults. A respiratory fluoroquinolone (moxifloxacin, gemifloxacin, levofloxacin) or a macrolide or doxycycline plus a beta-lactam are recommended if comorbid conditions are present. If MRSA is suspected, vancomycin (IV) or linezolid (IV, oral) should be added.

Omadacycline is a first-in-class aminomethylcycline that appears unaffected by the common resistance mechanisms (efflux pumps and ribosome protection) of older tetracyclines. It has demonstrated comparable efficacy and tolerability to contemporary antibiotics, including linezolid, for the treatment of ABSSSI, and a respiratory fluoroquinolone, for the treatment of CABP. Omadacycline may be an option to treat isolates that are resistant to conventional antibiotic classes. Its once-daily IV and oral dosing may ease the transition home for patients who require continued antibiotic therapy after hospital discharge.

## FDA APPROVAL TIMELINE

October 2, 2018

✓ Fast track ✓ Priority review ✓ Qualifed infectious diseases product

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $0 | $34 | $88 | $167 | $249 |

The forecast is a projection of total US sales per year.

# ulipristal acetate *oral*

Allergan

## PROPOSED INDICATIONS

Treatment of uterine fibroid-associated uterine bleeding

## CLINICAL OVERVIEW

Ulipristal is a selective progesterone receptor modulator (SPRM) that reversibly blocks progesterone receptors leading to fibroid cell death and reduced fibroid formation.

Pooled data from the pivotal VENUS-1 and VENUS-2 trials evaluated the effect of ulipristal on fibroid volume in premenopausal women (aged 18 to 50 years). After one 12-week treatment course of ulipristal 5 mg or 10 mg, respective changes in fibroid volume of -11.4% and -14.2% were reported, compared to an increase in volume by 6.8% with placebo. Continued reductions were seen after the second similar treatment course. Duration of effect was also reported during the 12-week treatment-free follow-up period (-16% and -31.5%, respectively). In VENUS-1, significantly more women achieved amenorrhea for ≥ 35 days during the 84-day treatment period (47.2% with 5 mg, 58.3% with 10 mg, 1.8% with placebo). In VENUS-2, significant improvements in Health-Related Quality of Life scores were reported with ulipristal compared to placebo. The most common adverse effects were hypertension, elevated serum creatinine phosphokinase, hot flushes, and nausea. No malignancies or endometrial atypical hyperplasia were reported. In addition, the European-based PEARL-2 study demonstrated non-inferiority of ulipristal to once-monthly, IM-administered leuprolide acetate for controlling uterine bleeding. Amenorrhea was achieved much sooner (5 to 7 days versus 21 days, respectively) and fewer hot flushes were reported with ulipristal.

## PLACE IN THERAPY

Uterine fibroids are benign growths in the uterus that affect up to 80% of women in the US ≤ 50 years of age. Uterine fibroids can cause pain and infertility, and 25% to 50% of affected women experience abnormal bleeding and decreased QOL. Current pharmacologic treatments include hormonal contraceptives and intrauterine devices (IUDs) that control bleeding. The gonadotropin-releasing hormone (GnRH) agonist, leuprolide (Lupron®), is the only treatment available that shrinks fibroids. Some procedures (e.g., myomectomy, hysteroscopy endometrial ablation, uterine artery embolization) can remove fibroids while preserving fertility, but they may be temporary solutions since they do not stop the development of new fibroids. Hysterectomy is performed when other options are no longer feasible or the fibroids are very large.

Once-daily oral ulipristal acetate is as effective in controlling uterine bleeding and may be better tolerated compared to monthly IM leuprolide. However, ulipristal's impact on fertility, its long-term effectiveness, and the need for continued use through menopause are unknown. Other drugs in phase 3 research for uterine fibroids include the SPRM vilaprisan, and the GnRH inhibitors elagolix, linzagolix, and relugolix.

Ulipristal 30 mg tablet (Ella®) is currently FDA-approved for emergency contraception. Ulipristal 5 mg tablet is approved in Europe and Canada for the treatment of uterine fibroid symptoms. Notably, in February 2018, the European Medicines Ageny (EMA) issued a Public Health Advisory following reports of serious liver injury with ulipristal 5 mg, including liver failure leading to transplantation. While the review is ongoing, the EMA advises no new patients should begin ulipristal treatment and liver function should be carefully monitored in women who are already taking the product. The FDA delayed the decision of Allergan's application for ulipristal to allow for a full review of the product.

## FDA APPROVAL TIMELINE

August 2018

## FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $3 | $26 | $53 | $78 | $98 |

The forecast is a projection of total US sales per year.

# Biosimilar Overview

## CLINICAL OVERVIEW

Biosimilars are very different from generic drugs in that they are not exact duplicates of their reference biologic product. The FDA approval process for biosimilars is designed to ensure that the biosimilar product is highly similar to the reference product without having any meaningful clinical differences.

Many controversies surround biosimilars but regulatory and litigation hurdles remain. The FDA has issued final and draft guidances. In February 2017, the Agency issued final guidance on the nonproprietary naming of biologic products, which also applies to biosimilars. The biological products must bear a core name, followed by a distinguishing 4-letter, lowercase, hyphenated suffix that is devoid of meaning. The FDA is still considering how to implement the nomenclature for previously-approved biosimilar products. The international nonproprietary name (INN) impacts interchangeability as it affects the pharmacists' ability to substitute an interchangeable biosimilar for the reference product. Although the Agency has not released its final guidance on interchangeability, several states have already enacted biosimilar substitution legislation. The FDA withdrew their September 2017 draft industry guidance on determining similarity of a proposed biosimilar product to its reference product to allow for further consideration of the most current and relevant scientific methods in evaluating analytical data. The Agency will focus on providing flexibility for efficient development of biosimilars while maintaining high scientific standards. In July 2018, the FDA finalized its guidance on labeling of biosimilars. The guidance pertains to prescribing information (PI) but does not contain specific recommendations on interchangeability in the labeling. The labeling guidance provides recommendations on how to include, identify, and differentiate the biosimilar versus the reference product in various sections of the PI. The basic premise remains that the originator product's safety and effectiveness can be relied upon for HCPs to make prescribing decisions. Therefore, a biosimilar should include relevant data from the originator in its PI.

Biosimilars are expected to receive full extrapolation for the eligible indications of the reference products without requiring additional trials. Nevertheless, as each biosimilar comes to market, it will likely need to be considered individually.

Insulins are historically regulated by the FDA as small molecules. Since the reference products are not deemed biologics by the FDA, any generics are technically branded competitors and are not considered biosimilars under the FDA's definition. In practice, however, *follow-on* insulins are regarded to be complex molecules and considered in the biosimilar space.

## PLACE IN THERAPY

The patents of several biologic drugs are set to expire in the next few years, opening up the US market for biosimilar entry; however, patent litigation has resulted in significant launch delays of FDA-approved biosimilars. In June 2017, the US Supreme Court issued 2 rulings: (1) allowing a biosimilar manufacturer to provide launch notice of commercial marketing to the originator manufacturer before or after FDA approval of the biosimilar product; and (2) eliminating any federal requirement for disclosure, also known as the "patent dance;" however, some states may mandate disclosure. These decisions may bring biosimilars to the market sooner and potentially create price competition in the market place.

In July 2018, the FDA unveiled its Biosimilar Action Plan (BAP), a series of 11 steps to encourage biosimilar market competition, some of which were previously announced or underway. BAP contains 4 key strategies: 1) improving biosimilar development and approval process; 2) maximizing scientific and regulatory clarity for sponsors; 3) effective communications for patients, clinicians, and payers; and 4) reducing unfair tactics that may delay market approval and entry. The BAP strives to promote access to biosimilar products and reduce healthcare costs.

To date, a total of 12 biosimilars have received FDA approval. Of these, only 4 have entered the market.

| APPROVED BIOSIMILARS | | | | |
|---|---|---|---|---|
| **Brand Name (Nonproprietary name)** | **Manufacturer** | **Approval Date** | **Commercially Available** | **Originator Product (Manufacturer)** |
| Zarxio® (filgrastim-sndz) | Sandoz | March 2015 | ✓ | Neupogen® (Amgen) |
| Inflectra® (infliximab-dyyb) | Pfizer/ Celltrion | April 2016 | ✓ | Remicade® (Janssen) |
| Erelzi™ (etanercept-szzs) | Sandoz | August 2016 | - | Enbrel® (Amgen) |
| Amjevita™ (adalimumab-atto) | Amgen | September 2016 | - | Humira® (Abbvie) |
| Renflexis™ (infliximab-abda) | Merck | May 2017 | ✓ | Remicade (Janssen) |
| Cyltezo® (adalimumab-adbm) | Boehringer Ingelheim | August 2017 | - | Humira (Abbvie) |
| Mvasi™ (bevacizumab-awwb) | Amgen | September 2017 | - | Avastin® (Genentech) |
| Ixifi™ (infliximab-qbtx)* | Pfizer | December 2017 | - | Remicade (Janssen) |
| Ogivri™ (trastuzumab-dkst) | Mylan | December 2017 | - | Herceptin® (Genentech) |
| Retacrit™ (epoetin alfa-epbx) | Pfizer/ Hospira | May 2018 | - | Epogen® (Amgen)<br>Procrit® (Janssen) |
| Fulphila™ (pegfilgrastim-jmdb) | Mylan | June 2018 | ✓ | Neulasta® (Amgen) |
| Nivestym™ (filgrastim-aafi) | Pfizer | July 2018 | - | Neupogen (Amgen) |

* Pfizer already has Inflectra on the market and has not announced plans to launch Ixifi.

Also available are Eli Lilly's Basaglar® insulin glargine, a follow-on agent to Sanofi's Lantus®, and Sanofi's Admelog® insulin lispro, approved as a follow-on product to Eli Lilly's Humalog®.

A host of factors will contribute to market acceptability and the potential success of biosimilars. Payers, pharmacies, prescribers, and patients each play a role in market adoption of biosimilars.

While < 2% of Americans use biologics, they account for almost 40% of all prescription drug spending. Moreover, they comprised 70% of growth in drug spending from 2010 to 2015. Not surprisingly, there is a growing body of evidence on predicted biologic spend and potential biosimilar savings. The global biologic market is projected to exceed $390 billion by 2020. The global biosimilar market is expected to grow from $5.95 billion in 2018 to $23.63 billion in 2023. An IMS Health analysis expects biosimilars to save the US and Europe's top 5 markets up to $110 billion by 2020. It is estimated that in the US biosimilars will cost 15% to 35% less than the originator product. The potential cost savings, however, can vary based on the market segment where brand contracts can play a role. A 2017 report by the RAND Corporation estimates a $54 billion cost savings from biosimilars between 2017 and 2026. In July 2018, an FDA analysis reported that if Americans had access to FDA-approved biosimilars in 2017, it would have resulted in $4.5 billion savings. A 2017 analysis by the Moran Company projects biosimilars can save the government an estimated $11.4 billion by 2027, but it would require the CMS to revise its reimbursement policy for biosimilars. In November 2017, CMS revised its reimbursement policy. The CMS has begun to issue a unique Healthcare Common Procedure Coding System (HCPCS) code to each individual biosimilar. Under this new rule, Medicare Part B will separately code and pay for biosimilars and no longer group them into a common payment code with originator agents. A June 2018 study by the Pacific Research Institute, forecasts annual savings of up to $465 million from increased use of biosimilars to replace a single biologic, for commercial payers and Medicare, based on an infliximab case study.

Biosimilar products may provide an opportunity to increase access to important biologic therapies that may increase survival and/or QOL for many patients with difficult-to-treat diseases, while also reducing costs.

## Blood Modifier
# adalimumab (GP2017) *SC*

Novartis/ Sandoz

GP2017 is a biosimilar to Abbvie's Humira, a tumor necrosis factor alpha (TNFα) blocker indicated for the treatment of autoimmune disorders including rheumatoid arthritis (RA), juvenile idiopathic arthritis (JIA), ankylosing spondylitis (AS), plaque psoriasis (PSO), psoriatic arthritis (PsA), Crohn's disease (CD) in adults and children, ulcerative colitis (UC), hidradenitis suppurativa (HS), and non-infectious uveitis.

### FDA APPROVAL TIMELINE
November 16, 2018

### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $13,752 | $15,192 | $16,356 | $17,450 | $17,938 |

The forecast is a projection of total US sales per year for the *branded product*.

## Blood Modifier
# filgrastim *IV, SC*

Adello and Apotex are seeking biosimilars to Amgen's Neupogen, a leukocyte growth factor indicated for use in patients with nonmyeloid malignancies receiving myelosuppressive anti-cancer drugs; following induction or consolidation chemotherapy for acute myeloid leukemia (AML); with nonmyeloid malignancies undergoing myeloablative chemotherapy followed by bone marrow transplantation; to mobilize autologous hematopoietic progenitor cells for collection by leukapheresis; with symptomatic congenital neutropenia, cyclic neutropenia, or idiopathic neutropenia; and acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome [HSARS]).

### FDA APPROVAL TIMELINE
Adello
Quarter 3, 2018

Apotex (Grastofil)
Pending

### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $254 | $209 | $181 | $160 | $142 |

The forecast is a projection of total US sales per year for the *branded product*.

## Diabetes
# insulin glargine *SC*

Lusduna Nexvue is a follow-on insulin to Sanofi's Lantus, a long-acting insulin indicated for the treatment of type 1 diabetes mellitus (T1DM) and type 2 diabetes mellitus (T2DM).

### FDA APPROVAL TIMELINE
Merck (Lusduna Nexvue)
Pending

- Lusduna Nexvue has met all required regulatory standards for follow-on insulins regarding clinical and nonclinical safety, efficacy, and quality, but litigation claiming patent infringement invoked an automatic stay on final FDA approval for up to 30 months or a court decision in favor of Merck, whichever comes sooner.

### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $2,135 | $1,731 | $1,369 | $1,087 | $926 |

The forecast is a projection of total US sales per year for the *branded product*.

## Blood Modifier
# pegfilgrastim *SC*

CHS-1701 and Lapelga are biosimilars to Amgen's Neulasta, a leukocyte growth factor indicated for use in patients with nonmyeloid malignancies receiving myelosuppressive anti-cancer drugs and patients acutely exposed to myelosuppressive doses of radiation (HSARS).

### FDA APPROVAL TIMELINE
Coherus (CHS-1701)
November 3, 2018

Apotex (Lapelga)
Pending

### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $3,797 | $3,234 | $2,779 | $2,463 | $2,129 |

The forecast is a projection of total US sales per year for the *branded product*.

## Oncology
# rituximab *IV*

Teva/ Celltrion

Truxima is a biosimilar to Genentech's Rituxan®, a CD20-directed cytolytic antibody indicated for the treatment of non-Hodgkin's lymphoma (NHL), chronic lymphocytic leukemia (CLL), rheumatoid arthritis (RA), and antineutrophil cytoplasmic antibodies-associated vasculitis.

### FDA APPROVAL TIMELINE
November 30, 2018

### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $4,087 | $3,428 | $2,716 | $2,252 | $1,890 |

The forecast is a projection of total US sales per year for the *branded product*.

## Oncology
# trastuzumab *IV*

Herzuma and SB3 are biosimilars to Genentech's Herceptin, a HER2/neu receptor antagonist indicated for the treatment of HER2-positive breast cancer and HER2-positive metastatic gastric or gastroesophageal junction adenocarcinoma.

### FDA APPROVAL TIMELINE
Merck/ Samsung Bioepis (SB3)
October 20, 2018

Teva/ Celltrion (Herzuma)
December 18, 2018

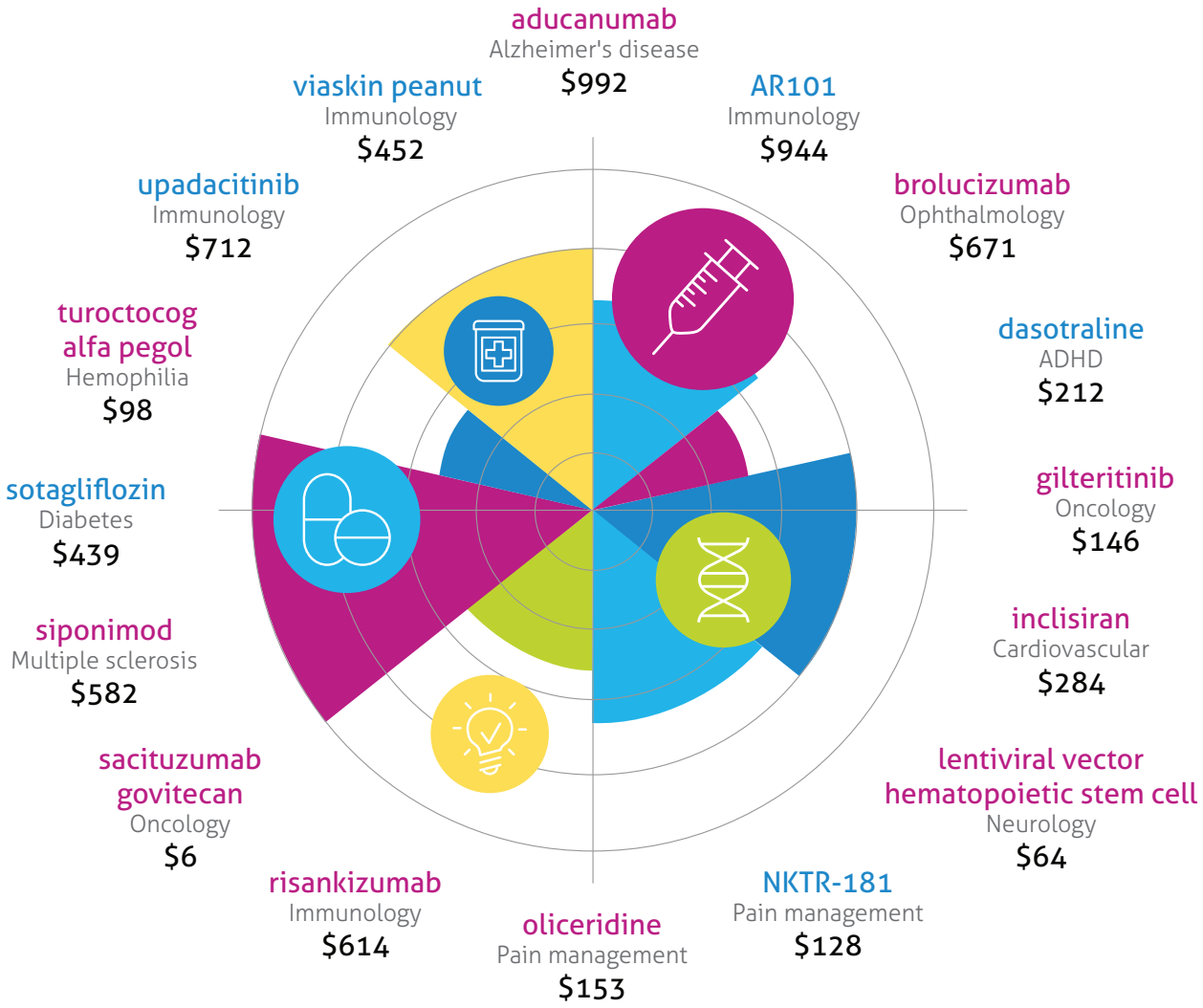
### FINANCIAL FORECAST (reported in millions)

| 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|
| $2,872 | $2,577 | $2,018 | $1,631 | $1,355 |

The forecast is a projection of total US sales per year for the *branded product*.

# Keep on Your Radar

Notable agents that are further from approval have been identified in this unique watch list. These are products with the potential for significant clinical and financial impact. Their development status is being tracked on the *MRx Pipeline* radar. These pipeline products, their respective class or proposed indication, as well as an estimated financial forecast for the year 2022, are displayed. The financials are projected total annual US sales, reported in *millions.*

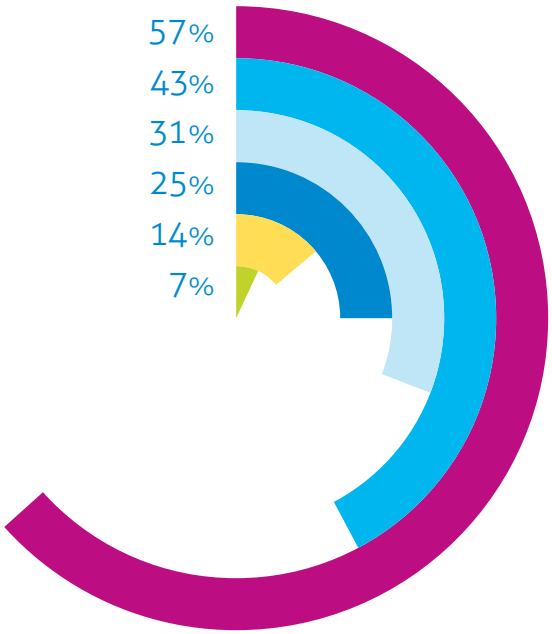



★ Specialty drug names appear in magenta throughout the publication.

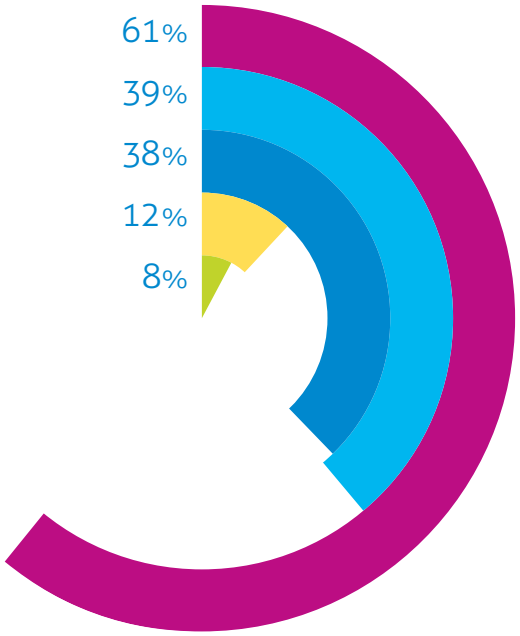
# Pipeline Drug List

The pipeline drug list is an aerial outline of drugs with anticipated FDA approval through 2019. It is not intended to be a comprehensive inventory of all drugs in the pipeline; emphasis is placed on drugs in high-impact categories. Investigational drugs with a Complete Response Letter (CRL) and those that have been withdrawn from development are also noted.

APPLICATION SUBMITTED TO THE FDA




IN PHASE 3 TRIALS




Specialty | Traditional | Priority review | Orphan drug | Breakthrough therapy | Biosimilar

★ Specialty drug names appear in magenta throughout the publication.

# PIPELINE DRUG LIST

★ Specialty drug names appear in magenta throughout the publication.

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| filgrastim (biosimilar to Amgen's Neupogen) | Adello | Neutropenia/ leukopenia | IV, SC | Submitted - BLA | Q3, 2018 |
| rizatriptan film | Redhill | Migraine treatment | SL | Submitted - 505(b)(2) NDA | H2, 2018 |
| tafenoquine succinate | 60 Degrees | Malaria prevention | Oral | Submitted - NDA; Fast track; Priority review | Jul-Aug 2018 |
| cocaine 4% and 10% topical solution | Lannett | Anesthesia | Topical | Submitted - 505(b)(2) NDA | 07/20/2018 |
| buprenorphine spray | Insys | Acute pain (moderate to severe) | SL | Submitted - 505(b)(2) NDA | 07/27/2018 |
| risperidone monthly depot | Indivior | Schizophrenia | SC | Submitted - 505(b)(2) NDA | 07/27/2018 |
| ultratrace Iobenguane I-131 | Progenics | Neuroendocrine tumors | IV | Submitted - NDA; Breakthrough therapy; Fast track; Orphan drug; Priority review | 07/30/2018 |
| cyclosporine | Sun | Dry eye | Intraocular | Submitted - 505(b)(2) NDA | August 2018 |
| ulipristal acetate | Allergan | Uterine fibroids | Oral | Submitted - 505(b)(2) NDA | August 2018 |
| canakinumab (Ilaris®) | Novartis | Atherosclerosis (secondary prevention) | SC | Submitted - sBLA | Aug-Oct 2018 |
| canagliflozin (Invokana®) | Janssen | CV risk reduction with T2DM | Oral | Submitted - sNDA | 08/02/2018 |
| canagliflozin/ metformin (Invokamet®/ Invokamet XR) | Janssen | CV risk reduction with T2DM | Oral | Submitted - sNDA | 08/02/2018 |
| elagolix | Abbvie | Endometriosis | Oral | Submitted - NDA; Priority review | 08/06/2018 |
| lumacaftor/ ivacaftor (Orkambi®) | Vertex | CF (ages 2-5 years) | Oral | Submitted - sNDA | 08/07/2018 |
| oxycodone | Pain Therapeutics | Chronic pain | Oral | Submitted - 505(b)(2) NDA | 08/07/2018 |
| methylphenidate DR | Highland | ADHD (adults, pediatrics) | Oral | Submitted - 505(b)(2) NDA | 08/08/2018 |
| aflibercept (Eylea®) - 12 week dosing | Regeneron | Wet AMD | Intraocular | Submitted - sBLA | 08/10/2018 |
| patisiran | Alnylam | Transthyretin (TTR)-related hereditary amyloidosis (Familial amyloid polyneuropathy) | IV | Submitted - NDA; Breakthrough therapy; Fast track; Orphan drug; Priority review | 08/10/2018 |
| migalastat | Amicus | Fabry disease | Oral | Submitted - NDA; Fast track; Orphan drug; Priority review | 08/13/2018 |
| ivacaftor (Kalydeco®) | Vertex | CF (ages 12 to < 24 months) | Oral | Submitted - sNDA | 08/15/2018 |
| nivolumab (Opdivo®) | Bristol-Myers Squibb | SCLC | IV | Submitted - sBLA; Priority review | 08/16/2018 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| stannsoporfin | Infacare | Hyperbilirubinemia | IM | Submitted - NDA; Fast track | 08/22/2018 |
| lanadelumab | Shire | Hereditary angioedema (prophylaxis; ages ≥ 12 years) | SC | Submitted - BLA; Breakthrough therapy; Fast track; Orphan drug; Priority review | 08/24/2018 |
| lenvatinib (Lenvima®) | Eisai | HCC (1st-line) | Oral | Submitted - sNDA; Orphan drug | 08/24/2018 |
| loteprednol etabonate 1% | Kala | Ocular pain and inflammation after ophthalmic surgery | Ophthalmic | Submitted - 505(b)(2) NDA | 08/24/2018 |
| lusutrombopag | Shionogi | Thrombocytopenia | Oral | Submitted - NDA; Fast track; Priority review | 08/24/2018 |
| alirocumab (Praluent®) | Regeneron | Hypercholesterolemia (with apheresis) | SC | Submitted - sBLA | 08/27/2018 |
| tretinoin 0.05% lotion | Valeant | Acne | Topical | Submitted - 505(b)(2) NDA | 08/27/2018 |
| eravacycline | Tetraphase | Intra-abdominal infections | IV, Oral | Submitted - NDA; Fast track; Priority review | 08/28/2018 |
| lorlatinib | Pfizer | NSCLC (ALK+) | Oral | Submitted - NDA; Breakthrough therapy; Orphan drug; Priority review | 08/29/2018 |
| volanesorsen | Akcea | Familial chylomicronemia syndrome | SC | Submitted - NDA; Orphan drug | 08/30/2018 |
| damoctocog alfa pegol | Bayer | Hemophilia A | IV | Submitted - BLA | 08/31/2018 |
| dasotraline | Sunovion | ADHD (adults, pediatrics) | Oral | Submitted - NDA | 08/31/2018 |
| clobazam oral film | Aquestive | Lennox-Gastaut syndrome | SL | Submitted - 505(b)(2) NDA | September 2018 |
| dacomitinib | Pfizer | NSCLC (1st-line, locally advanced, EGFR+) | Oral | Submitted - NDA; Orphan drug; Priority review | September 2018 |
| eltrombopag (Promacta®) | Novartis | Aplastic anemia (severe, 1st-line, combination with standard therapy) | Oral | Submitted - sNDA; Breakthrough therapy; Orphan drug; Priority review | Sep-Oct 2018 |
| moxetumomab pasudotox | AstraZeneca | Hairy cell leukemia | IV | Submitted - BLA; Priority review | Sep-Oct 2018 |
| epinephrine 0.15 mg (Symjepi®) | Adamis | Anaphylaxis (pediatrics) | SC | Submitted - 505(b)(2) sNDA | 09/03/2018 |
| mogamulizumab | Amgen | Cutaneous T cell lymphoma | IV | Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review | 09/04/2018 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| atezolizumab (Tecentriq®) | Genentech | NSCLC (1st-line, in combination with chemotherapy) | IV | Submitted - sBLA; Priority review | 09/05/2018 |
| mepolizumab (Nucala) | GlaxoSmithKline | COPD (eosinophilic phenotype; maintenance) | SC | Submitted - sBLA | 09/07/2018 |
| fremanezumab | Teva | Migraine prevention | IV, SC | Submitted - BLA; Fast track; Priority review | 09/14/2018 |
| C1 esterase inhibitor, recombinant (Ruconest®) | Pharming | Hereditary angioedema (routine prophylaxis) | IV | Submitted - sNDA; Fast track; Orphan drug | 09/21/2018 |
| pembrolizumab (Keytruda) | Merck | NSCLC (1st-line, in combination with pemetrexed and platinum chemotherapy) | IV | Submitted - sBLA; Priority review | 09/21/2018 |
| amikacin (liposomal) | Insmed | Nontuberculous mycobacterial lung disease | Inhaled | Submitted - NDA; Breakthrough therapy; Fast track; Orphan drug; Priority review; Qualified infectious diseases product | 09/28/2018 |
| galcanezumab | Eli Lilly | Migraine prevention | SC | Submitted - BLA; Fast track | 09/28/2018 |
| perampanel (Fycompa®) | Eisai | Partial onset seizures (ages 2-11 years) | Oral | Submitted - sNDA; Priority review | 09/28/2018 |
| testosterone auto-injector | Antares | Hypogonadism | SC | Submitted - 505(b)(2) NDA | 09/28/2018 |
| levonorgestrel-releasing intrauterine system (Liletta®) | Allergan | Contraception (up to 5 years) | Intravaginal | Submitted - sNDA | Q4, 2018 |
| ibrutinib (Imbruvica®) | Abbvie | Waldenstrom macroglobulinemia (in combination with rituximab) | Oral | Submitted - sNDA; Orphan drug; Priority review | October 2018 |
| sarecycline | Allergan | Acne | Oral | Submitted - NDA | October 2018 |
| nestorone/ ethinyl estradiol contraceptive vaginal ring (1-year) | Allergan | Contraception | Intravaginal | Submitted - NDA | Oct-Nov 2018 |
| omadacycline | Paratek | ABSSSI; Community-aquired bacterial pneumonia (CABP) | IV, Oral | Submitted - NDA; Fast track; Priority review; Qualified infectious diseases product | 10/02/2018 |
| emicizumab-kxwh (Hemlibra®) | Genentech | Hemophilia A (without inhibitors) | SC | Submitted - sBLA; Breakthrough therapy; Orphan drug; Priority review | 10/04/2018 |
| amisulpride | Acacia | Post-operative nausea/ vomiting | IV | Submitted - NDA | 10/05/2018 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| duvelisib | Verastem | CLL/ SLL; Follicular lymphoma | Oral | Submitted - NDA; Fast track; Orphan drug; Priority review | 10/05/2018 |
| halobetasol propionate | Valeant | PSO | Topical | Submitted - 505(b)(2) NDA | 10/05/2018 |
| inotersen | Ionis/ Akcea | Transthyretin (TTR)-related hereditary amyloidosis (Familial amyloid polyneuropathy) | SC | Submitted - NDA; Fast track; Orphan drug; Priority review | 10/05/2018 |
| levodopa | Acorda | Parkinson's disease | Inhaled | Submitted - 505(b)(2) NDA | 10/05/2018 |
| rivaroxaban 2.5 mg (Xarelto®) twice daily | Janssen | Coronary artery disease; Peripheral arterial disease | Oral | Submitted - sNDA; Fast track | 10/11/2018 |
| dupilumab (Dupixent®) | Regeneron | Asthma (severe, uncontrolled) | SC | Submitted - sBLA | 10/20/2018 |
| trastuzumab (biosimilar to Genentech's Herceptin) | Merck/ Samsung Bioepis | Breast cancer | IV | Submitted - BLA | 10/20/2018 |
| doravirine | Merck | HIV-1 infection | Oral | Submitted - NDA | 10/23/2018 |
| doravirine/ lamivudine/ tenofovir disoproxil fumarate | Merck | HIV-1 infection | Oral | Submitted - NDA | 10/23/2018 |
| cemiplimab | Regeneron | Cutaneous squamous cell carcinoma | IV | Submitted - BLA; Breakthrough therapy; Priority review | 10/26/2018 |
| sodium oxybate (Xyrem®) | Jazz | Narcolepsy-related cataplexy (pediatrics) | Oral | Submitted - sNDA; Priority review | 10/26/2018 |
| estradiol/ progesterone | TherapeuticsMD | Menopausal-related vasomotor symptoms | Oral | Submitted - 505(b)(2) NDA | 10/28/2018 |
| pembrolizumab (Keytruda) | Merck | Squamous NSCLC (1st-line, in combination with carbolatin-paclitaxel or nab-paclitaxal, regardless of PD-L1 expression) | IV | Submitted - sBLA; Priority review | 10/30/2018 |
| pasireotide diaspartate long-acting (Signifor® LAR) | Novartis | Cushing's disease | IM, SC | Submitted - sNDA | November 2018 |
| oliceridine | Trevena | Acute pain (moderate to severe) | IV | Submitted - NDA; Breakthrough therapy; Fast track | 11/02/2018 |
| sufentanil | Acelrx | Acute pain (adults, medically supervised settings) | SL | Submitted - 505(b)(2) NDA | 11/02/2018 |
| pegfilgrastim (biosimilar for Amgen's Neulasta) | Coherus | Neutropenia/ leukopenia | SC | Submitted - BLA | 11/03/2018 |
| fluocinolone acetonide (Iluvien®) | Alimera | Uveitis | Intraocular | Submitted - sNDA; Orphan drug | 11/05/2018 |
| pembrolizumab (Keytruda) | Merck | HCC (including secondary metastases) | IV | Submitted - sBLA; Priority review | 11/09/2018 |
| revefenacin | Theravance | COPD | Inhaled | Submitted - NDA | 11/13/2018 |
| adalimumab (biosimilar to Abbvie's Humira) | Novartis/ Sandoz | RA; AS; PSO; PsA; JIA; CD; UC | SC | Submitted - BLA | 11/16/2018 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| rifamycin | Cosmo | Traveler's diarrhea | Oral | Submitted - NDA; Fast track; Priority review; Qualified infectious diseases product | 11/16/2018 |
| emapalumab | Novimmune | Hemophagocytic lymphohistiocytosis | IV | Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review | 11/20/2018 |
| larotrectinib | Loxo Oncology | Solid tumors (locally advanced or metastatic tumors with neurotrophic tyrosine receptor kinase [NTRK] gene fusion) | Oral | Submitted - NDA; Breakthrough therapy; Orphan drug; Priority review; Rare pediatric disease product | 11/26/2018 |
| amifampridine | Catalyst | Lambert-Eaton myasthenic syndrome | Oral | Submitted - NDA; Breakthrough therapy; Orphan drug; Priority review | 11/28/2018 |
| gilteritinib | Astellas | AML (FLT3+) | Oral | Submitted - NDA; Fast track; Orphan drug; Priority review | 11/29/2018 |
| bupivacaine collagen matrix implant | Innocoll | Postsurgical pain | Implant | Submitted - NDA | 11/30/2018 |
| rituximab (biosimilar to Genentech's Rituxan) | Teva/ Celltrion | CLL/ SLL; RA; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis | IV | Submitted - BLA | 11/30/2018 |
| glasdegib | Pfizer | AML (adults, treatment-naïve) | Oral | Submitted - NDA; Orphan drug; Priority review | December 2018 |
| talazoparib | Pfizer | Breast cancer (BRCA+, HER2-, locally advanced or metastatic) | Oral | Submitted - NDA; Priority review | December 2018 |
| itraconazole | Hedgepath | Fungal infections (systemic) | Oral | Submitted - 505(b)(2) NDA | Dec 2018 - Jan 2019 |
| siponimod | Novartis | MS (secondary progressive) | Oral | Submitted - NDA; Priority review | Dec 2018 - Feb 2019 |
| trastuzumab (biosimilar to Genentech's Herceptin) | Teva/ Celltrion | Breast cancer | IV | Submitted - BLA | 12/18/2018 |
| brexanolone | Sage | Postpartum depression | IV | Submitted - NDA; Breakthrough therapy; Priority review | 12/19/2018 |
| solriamfetol | Jazz | Narcolepsy; Sleep apnea | Oral | Submitted - NDA; Orphan drug | 12/20/2018 |
| calaspargase pegol | Shire | ALL | IV | Submitted - BLA | 12/21/2018 |
| prucalopride | Shire | Chronic idiopathic constipation | Oral | Submitted - NDA | 12/21/2018 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| baloxavir marboxil | Genentech | Influenza treatment | Oral | Submitted - NDA; Priority review | 12/24/2018 |
| buprenorphine depot | Braeburn | Substance use disorder | SC | Submitted - 505(b)(2) NDA; Fast track | 12/26/2018 |
| glycerol phenylbutyrate (Ravicti®) | Horizon | Urea cycle disorders (infants < 2 months of age) | Oral | Submitted - sNDA; Fast track; Orphan drug | 12/27/2018 |
| astodrimer sodium | Starpharma | Bacterial vaginosis (treatment & prevention) | Intravaginal | Submitted - NDA; Fast track; Priority review; Qualified infectious diseases product | 12/28/2018 |
| dexamethasone punctum plug | Ocular Therapeutics | Ocular pain folllowing ophthalmic surgery | Intraocular | Submitted - NDA | 12/28/2018 |
| pembrolizumab (Keytruda) | Merck | SCCHN (full approval) | IV | Submitted - sBLA | 12/28/2018 |
| ospemifene (Osphena®) | Duchesnay | Postmenopausal-related vaginal dryness | Oral | Submitted - sNDA | January 2019 |
| tacrolimus (Envarsus XR®) | Veloxis | Kidney transplant rejection | Oral | Submitted - sNDA; Orphan drug | 01/07/2019 |
| romosozumab | Amgen | Osteoporosis (women) | SC | Submitted - sBLA | 01/11/2019 |
| cabozantinib (Cabometyx®) | Exelixis | HCC | Oral | Submitted - sNDA; Orphan drug | 01/14/2019 |
| sacituzumab govitecan | Immunomedics | Breast cancer (metastatic, triple-negative) | IV | Submitted - BLA; Breakthrough therapy; Fast track; Priority Review | 01/18/2019 |
| influenza quadrivalent vaccine (Fluzone®) | Sanofi | Influenza prevention (aged 6-35 months) | IM | Submitted - sBLA | 01/28/2019 |
| apomorphine | Sunovion | Parkinson's disease (on-demand treatment of all types of motor "off" episodes) | SL | Submitted - 505(b)(2) NDA; Fast track | 01/29/2019 |
| samidorphan/ buprenorphine | Alkermes | MDD | SL | Submitted - NDA; Fast track | 01/31/2019 |
| iclaprim | Motif Bio | ABSSSI | IV | Submitted - NDA; Fast track; Qualified infectious diseases product | 02/14/2019 |
| pembrolizumab (Keytruda) | Merck | Melanoma (adjunctive, resected, high-risk stage 3) | IV | Submitted - sBLA; Breakthrough therapy; Orphan drug | 02/15/2019 |
| ravulizumab | Alexion | Paroxysmal nocturnal hemoglobinuria | IV | Submitted - BLA; Orphan drug; Priority review | 02/19/2019 |
| nivolumab (Opdivo) | Bristol-Myers Squibb | NSCLC (1st-line, in combination with ipilimumab) | IV | Submitted - sBLA; Fast track | 02/20/2019 |
| afamelanotide | Clinuvel | Erythropoietic protoporphyria | Intradermal | Submitted - NDA; Fast track; Orphan drug | 02/25/2019 |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| loteprednol etabonate 0.38% gel (submicron) | Bausch & Lomb | Ocular pain and inflammation after ophthalmic surgery | Ophthalmic | Submitted - 505(b)(2) NDA | 02/25/2019 |
| tagraxofusp | Stemline | Blastic plasmacytoid dendritic cell neoplasm | IV | Submitted - BLA; Breakthrough therapy; Orphan drug | 02/25/2019 |
| turoctocog alfa pegol | Novo Nordisk | Hemophilia A | IV | Submitted - BLA | 02/27/2019 |
| macitentan (Opsumit®) | Janssen | Chronic thromboembolic pulmonary hypertension (inoperable) | Oral | Submitted - sNDA | 02/28/2019 |
| netarsudil/ latanoprost | Aerie | Glaucoma/ ocular hypertension | Ophthalmic | Submitted - 505(b)(2) NDA | 03/15/2019 |
| bremelanotide | AMAG | Female sexual arousal disorder | SC | Submitted - NDA | 03/22/2019 |
| olopatadine/ mometasone furoate | Glenmark | Allergic rhinitis | Intranasal | Submitted - 505(b)(2) NDA | 03/22/2019 |
| sotagliflozin | Sanofi | T1DM; T2DM | Oral | Submitted - NDA | 03/26/2019 |
| aclidinium bromide (Tudorza® Pressair®) | Circassia | CV outcomes | Inhaled | Submitted - sNDA | 04/01/2019 |
| aclidinium/ formoterol | Circassia | COPD | Inhaled | Submitted - NDA | 04/01/2019 |
| metoclopramide spray | Evoke | Diabetic gastroparesis | Intranasal | Submitted - 505(b)(2) NDA | 04/01/2019 |
| sumatriptan | Dr. Reddy's | Migraine treatment | Intranasal | Submitted - 505(b)(2) NDA | 04/02/2019 |
| risankizumab | Abbvie | PSO | SC | Submitted - BLA | 04/25/2019 |
| trastuzumab SC (Herceptin) | Genentech | Breast cancer | SC | Submitted - BLA | May 2019 |
| tesamorelin single-vial formulation (Egrifta®) | Theratechnologies | HIV lipodystrophy | SC | Submitted - sNDA | 05/03/2019 |
| venetoclax (Venclexta®) | Abbvie | AML | Oral | Submitted - sNDA; Breakthrough therapy; Orphan drug | 05/10/2019 |
| NKTR-181 | Nektar | Chronic low back pain | Oral | Submitted - NDA; Fast track | 05/31/2019 |
| filgrastim (biosimilar to Amgen's Neupogen) | Apotex | Neutropenia/ leukopenia | IV, SC | Submitted - BLA | Pending |
| insulin glargine (follow-on to Sanofi's Lantus) | Merck | T1DM; T2DM | SC | Submitted - 505(b)(2) NDA | Pending |
| pegfilgrastim (biosimilar for Amgen's Neulasta) | Apotex | Neutropenia/ leukopenia | SC | Submitted - BLA | Pending |
| plasminogen (human) | Prometic Life | Hypoplasminogenemia | IV | Submitted - BLA; Fast track; Orphan drug; Priority review; Rare pediatric disease product | Pending |
| abatacept (Orencia®) | Bristol-Myers Squibb | Dermatomyositis; Lupus nephritis; Sjogren's syndrome | IV, SC | Phase 3 - sBLA; Orphan drug | TBD |
| acalabrutinib (Calquence®) | AstraZeneca | CLL/ SLL | Oral | Phase 3 - sNDA; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| adalimumab (biosimilar to Abbvie's Humira) | Coherus | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| adalimumab (biosimilar to Abbvie's Humira) | Fresenius | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| adalimumab (biosimilar to Abbvie's Humira) | Kyowa Hakko Kirin | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| adalimumab (biosimilar to Abbvie's Humira) | Momenta | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| adalimumab (biosimilar to Abbvie's Humira) | Mylan/ Biocon | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| adalimumab (biosimilar to Abbvie's Humira) | Pfizer | RA; AS; PSO; PsA; JIA; CD; UC | SC | Phase 3 - BLA | TBD |
| aducanumab | Biogen | Alzheimer's disease | IV | Phase 3 - BLA; Fast track | TBD |
| aldoxorubicin | Nantworks | Sarcoma | IV | Phase 3 - 505(b)(2) NDA; Orphan drug | TBD |
| alicaforsen sodium | Atlantic Healthcare | UC | Rectal | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| alirocumab (Praluent) | Regeneron | CV outcomes | SC | Phase 3 - sBLA | TBD |
| allogenic expanded adipose-derived stem cells | Tigenix | CD (fistulizing) | IV | Phase 3 - BLA; Orphan drug | TBD |
| alpelisib | Novartis | Breast cancer | Oral | Phase 3 - NDA | TBD |
| amifampridine | Catalyst | Myasthenia gravis; Congenital myasthenic syndrome | Oral | Phase 3 - NDA; Orphan drug | TBD |
| amikacin (liposomal) | Insmed | CF | Inhaled | Phase 3 - NDA; Orphan drug | TBD |
| amrubicin | Celgene | SCLC | IV | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| andecaliximab | Gilead | Gastric cancer | IV | Phase 3 - BLA; Orphan drug | TBD |
| andolast | Mylan | Asthma (atopic) | Inhaled | Phase 3 - NDA | TBD |
| anifrolumab | AstraZeneca | SLE | IV | Phase 3 - BLA; Fast track | TBD |
| anlotinib | Advenchen | Sarcoma | Oral | Phase 3 - NDA; Orphan drug | TBD |
| anti-digoxin antibody | AMAG | Eclampsia/ pre-eclampsia | IV | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| apalutamide (Erleada®) | Janssen | Prostate cancer (metastatic, castration resistant, 1st-line) | Oral | Phase 3 - sNDA | TBD |
| apremilast (Otezla®) | Celgene | Axial spondyloarthritis; Behçet syndrome | Oral | Phase 3 - sNDA; Orphan drug | TBD |
| AR101 | Aimmune | Peanut allergy | Oral | Phase 3 - BLA; Breakthrough therapy; Fast track | TBD |
| atezolizumab (Tecentriq) | Genentech | Melanoma; RCC; Breast cancer; CRC; Ovarian cancer; SCLC; Prostate cancer; HCC | IV | Phase 3 - sBLA; Breakthrough therapy; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| avacopan | Chemocentryx | Antineutrophil cytoplasmic antibodies associated vasculitis | Oral | Phase 3 - NDA; Orphan drug | TBD |
| avatrombopag (Doptelet®) | Dova | Idiopathic thrombocytopenic purpura | Oral | Phase 3 - sNDA | TBD |
| avelumab (Bavencio®) | Merck | DLBCL; Gastric cancer; NSCLC; Ovarian cancer; RCC; SCCHN | IV | Phase 3 - sBLA; Breakthrough therapy | TBD |
| AVXS-101 | Avexis | Spinal muscular atrophy | IV | Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| axalimogene filolisbac | Advaxis | Cervical cancer | IV | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| baclofen/ naltrexone/ sorbitol | Pharnext | Charcot-Marie-Tooth disease | Oral | Phase 3 - NDA; Orphan drug | TBD |
| baricitinib (Olumiant®) | Eli Lilly | Atopic dermatitis | Oral | Phase 3 - sNDA | TBD |
| bempedoic acid | Esperion | Dyslipidemia | Oral | Phase 3 - NDA | TBD |
| bempedoic acid/ ezetimibe | Esperion | Dyslipidemia | Oral | Phase 3 - NDA | TBD |
| benralizumab (Fasenra®) | AstraZeneca | Nasal polyposis | SC | Phase 3 - sBLA | TBD |
| bevacizumab (biosimilar to Genentech's Avastin) | Boehringer Ingelheim | CRC; NSCLC; Ovarian/ fallopian tube/ peritoneal cancer; Glioblastoma; RCC | IV | Phase 3 - BLA | TBD |
| bevacizumab (biosimilar to Genentech's Avastin) | Centus | CRC; NSCLC; Ovarian/ fallopian tube/ peritoneal cancer; Glioblastoma; RCC | IV | Phase 3 - BLA | TBD |
| bevacizumab (biosimilar to Genentech's Avastin) | Mylan/ Biocon | CRC; NSCLC; Ovarian/ fallopian tube/ peritoneal cancer; Glioblastoma; RCC | IV | Phase 3 - BLA | TBD |
| bevacizumab (biosimilar to Genentech's Avastin) | Pfizer | CRC; NSCLC; Ovarian/ fallopian tube/ peritoneal cancer; Glioblastoma; RCC | IV | Phase 3 - BLA | TBD |
| brexpiprazole (Rexulti®) | Otsuka | Alzheimer's disease; Bipolar disorder | Oral | Phase 3 - sNDA; Fast track | TBD |
| brincidofovir | Chimerix | Adenovirus infection | Oral | Phase 3 - NDA; Fast track | TBD |
| brolucizumab | Novartis | Wet AMD; Diabetic macular edema | Intraocular | Phase 3 - BLA | TBD |
| budesonide/ formoterol | AstraZeneca | COPD | Inhaled | Phase 3 - NDA | TBD |
| budesonide/ glycopyrronium/ formoterol | AstraZeneca | COPD | Inhaled | Phase 3 - NDA | TBD |
| bupivacaine and meloxicam | Heron | Postsurgical pain | Instillation | Phase 3 - NDA; Breakthrough therapy; Fast track | TBD |
| C1-esterase inhibitor (Cinryze®) | Shire | Hereditary angioedema (adults & pediatrics) | SC | Phase 3 - sBLA | TBD |
| canagliflozin (Invokana) | Janssen | Diabetic nephropathy | Oral | Phase 3 - sNDA | TBD |
| cannabidiol (Epidiolex®) | GW | Infantile spasms (West syndrome) | Oral | Phase 3 - sNDA; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| cannabidiol oral solution | Insys | Dravet syndrome; Lennox-Gastaut syndrome; Infantile spasms (West syndrome) | Oral | Phase 3 - NDA; Orphan drug | TBD |
| caplacizumab | Ablynx | Thrombotic thrombocytopenic purpura | IV | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| capsaicin | Centrexion | Arthritis pain (knee osteoarthritis) | Intra-articular | Phase 3 - NDA; Fast track | TBD |
| carotuximab | Tracon | Sarcoma | IV | Phase 3 - BLA; Orphan drug | TBD |
| cediranib | AstraZeneca | Ovarian cancer; Biliary tract cancer | Oral | Phase 3 - NDA; Orphan drug | TBD |
| cefiderocol | Shionogi | HAP (bacterial) | IV | Phase 3 - NDA | TBD |
| celiprolol | Acer | Vascular Ehlers-Danlos syndrome | Oral | Phase 3 - NDA; Orphan drug | TBD |
| cemiplimab | Regeneron | Cervical cancer; NSCLC | IV | Phase 3 - BLA | TBD |
| cetirizine | Pfizer | Urticaria | IV | Phase 3 - 505(b)(2) NDA | TBD |
| citrulline | Asklepion | Acute respiratory distress syndrome (ARDS) | IV | Phase 3 - NDA; Orphan drug | TBD |
| cortexolone 17a-propionate | Cassiopea | Acne | Topical | Phase 3 - NDA | TBD |
| CTP-modified human growth hormone | Opko Health | Growth hormone deficiency | SC | Phase 3 - BLA; Orphan drug | TBD |
| cyclobenzaprine | Tonix | Post-traumatic stress disorder | Oral, SL | Phase 3 - 505(b)(2) NDA; Breakthrough therapy; Fast track | TBD |
| dapagliflozin (Farxiga®) | AstraZeneca | T1DM; Diabetic neuropathy; Renal and CV outcomes in patients with CKD | Oral | Phase 3 - sNDA | TBD |
| daprodustat | GlaxoSmithKline | Anemia due to CKD (dialysis dependent & independent) | Oral | Phase 3 - NDA | TBD |
| darleukin | Philogen | Melanoma | IV | Phase 3 - BLA | TBD |
| dehydrated human amnion-chorion membrane | Mimedx | Achilles tendonitis; Plantar fasciitis | Injection | Phase 3 - BLA | TBD |
| denileukin diftitox (Ontak®) | Dr. Reddy's | Peripheral T cell lymphoma | IV | Phase 3 - sBLA | TBD |
| derazantinib | Arqule | Biliary tract cancer | Oral | Phase 3 - NDA; Orphan drug | TBD |
| dexamethasone | Eyegate | Uveitis | Intraocular | Phase 3 - 505(b)(2) NDA | TBD |
| dexamethasone SR | Otonomy | Meniere's disease | Intratympanic | Phase 3 - 505(b)(2) NDA; Fast track | TBD |
| dianhydrogalactitol | Delmar | Glioblastoma (recurrent) | IV | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| difelikefalin | Cara | Pruritus related to CKD | IV | Phase 3 - NDA; Breakthrough therapy | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| dinutuximab beta | EUSA | Neuroblastoma | SC | Phase 3 - BLA; Orphan drug | TBD |
| diroximel fumarate | Alkermes | MS | Oral | Phase 3 - 505(b)(2) NDA | TBD |
| docosahexaenoic acid | Sancilio | Sickle cell anemia | Oral | Phase 3 - 505(b)(2) NDA; Orphan drug | TBD |
| dolutegravir/ lamivudine | GlaxoSmithKline | HIV-1 infection | Oral | Phase 3 - NDA | TBD |
| donor lymphocytes depleted alloreactive T cells | Kiadis | AML | IV | Phase 3 - BLA | TBD |
| dupilumab (Dupixent) | Regeneron | Nasal polyposis | SC | Phase 3 - sBLA | TBD |
| durvalumab (Imfinzi®) | AstraZeneca | HCC; SCLC; SCCHN | IV | Phase 3 - sBLA; Fast track | TBD |
| dusquetide | Soligenix | Mucositis | IV | Phase 3 - NDA; Fast track | TBD |
| eculizumab (Soliris®) | Alexion | Neuromyelitis optica (Devic's syndrome); Delayed graft function | IV | Phase 3 - sBLA; Orphan drug | TBD |
| eflapegrastim | Spectrum | Neutropenia/ leukopenia | SC | Phase 3 - BLA | TBD |
| efpeglenatide | Hanmi | T2DM | SC | Phase 3 - NDA | TBD |
| elafibranor | Genfit | NASH | Oral | Phase 3 - NDA; Fast track | TBD |
| elagolix | Abbvie | Uterine fibroids | Oral | Phase 3 - NDA | TBD |
| epoetin alfa (biosimilar to Janssen's Procrit) | Novartis | Anemia due to CKD (dialysis dependent & independent) | IV, SC | Phase 3 - BLA | TBD |
| epratuzumab | Immunomedics | ALL | IV | Phase 3 - BLA; Orphan drug | TBD |
| eptinezumab | Alder | Migraine prevention | SC | Phase 3 - BLA | TBD |
| erdosteine | Alitair | COPD | Oral | Phase 3 - NDA | TBD |
| esketamine | Janssen | MDD | Intranasal | Phase 3 - NDA; Breakthrough therapy; Fast track | TBD |
| etanercept (biosimilar to Amgen's Enbrel) | Coherus | RA; JIA; AS; PSO; PsA | SC | Phase 3 - BLA | TBD |
| etanercept (biosimilar to Amgen's Enbrel) | Merck/ Samsung Bioepis | RA; JIA; AS; PSO; PsA | SC | Phase 3 - BLA | TBD |
| etrolizumab | Genentech | CD; UC | IV, SC | Phase 3 - BLA; Orphan drug | TBD |
| favipiravir | Medivector | Influenza treatment | Oral | Phase 3 - NDA | TBD |
| fenfluramine (low-dose) | Zogenix | Dravet syndrome; Lennox-Gastaut syndrome | Oral | Phase 3 - NDA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| ferric maltol | Shield | Anemia due to CKD (dialysis-independent) | Oral | Phase 3 - NDA | TBD |
| fevipiprant | Novartis | Asthma (severe, uncontrolled) | Oral | Phase 3 - NDA | TBD |
| filgotinib | Gilead | RA; CD; UC | Oral | Phase 3 - NDA | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®) | GlaxoSmithKline | Asthma | Inhaled | Phase 3 - sNDA | TBD |
| formoterol fumarate | AstraZeneca | COPD | Inhaled | Phase 3 - 505(b)(2) NDA | TBD |
| fosfomycin | Zavante | Complicated UTI | IV | Phase 3 - 505(b)(2) NDA; Fast track; Qualified infectious diseases product | TBD |
| fosmetpantotenate | Retrophin | Pantothenate kinase-associated neurodegeneration | IV | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| fostemsavir | GlaxoSmithKline | HIV-1 infection | Oral | Phase 3 - NDA; Breakthrough therapy; Fast track | TBD |
| fremanezumab | Teva | Cluster headache prevention | SC | Phase 3 - BLA; Fast track | TBD |
| fusidic acid | Cempra | ABSSSI; Bone and joint infections | Oral | Phase 3 - NDA; Orphan drug; Qualified infectious diseases product | TBD |
| galcanezumab | Eli Lilly | Cluster headache prevention | SC | Phase 3 - BLA; Fast track | TBD |
| gefapixant | Merck | Chronic cough | Oral | Phase 3 - NDA | TBD |
| givosiran | Alnylam | Porphyria | SC | Phase 3 - NDA; Breakthrough therapy; Orphan drug | TBD |
| glycopyrrolate hydrofluoroalkane (metered dose inhaler) | AstraZeneca | COPD | Inhaled | Phase 3 - NDA | TBD |
| glycopyrronium bromide (Seebri™ Neohaler®) | Sumitomo Dainippon | Asthma | Inhaled | Phase 3 - sNDA | TBD |
| golodirsen | Sarepta | Duchenne muscular dystrophy | IV | Phase 3 - NDA | TBD |
| grazoprevir/ elbasvir (Zepatier®) | Merck | HCV infection (with CKD) | Oral | Phase 3 - sNDA; Breakthrough therapy | TBD |
| GS010 | Gensight | Leber's hereditary optic neuropathy | Intraocular | Phase 3 - BLA; Orphan drug | TBD |
| human plasminogen | Kedrion | Ligneous conjunctivitis | Topical | Phase 3 - BLA; Orphan drug | TBD |
| ibritumomab tiuxetan | Spectrum | DLBCL | IV | Phase 3 - BLA | TBD |
| icosapent ethyl (Vascepa®) | Amarin | Major CV event risk reduction | Oral | Phase 3 - sNDA | TBD |
| idasanutlin | Genentech | AML | Oral | Phase 3 - NDA | TBD |
| idebenone | Santhera | Duchenne muscular dystrophy | Oral | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| inclisiran | The Medicines Company | Dyslipidemia | SC | Phase 3 - NDA; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| indacaterol/ glycopyrronium bromide/ mometasone furoate | Novartis | Asthma | Inhaled | Phase 3 - NDA | TBD |
| indacaterol/ mometasone furoate | Novartis | Asthma | Inhaled | Phase 3 - NDA | TBD |
| inebilizumab | AstraZeneca | Neuromyelitis optica (Devic's syndrome) | IV | Phase 3 - BLA; Orphan drug | TBD |
| infliximab (biosimilar to Janssen's Remicade) | Amgen | RA | IV | Phase 3 - BLA | TBD |
| insulin glargine (follow-on to Sanofi's Lantus) | Gan & Lee | T1DM; T2DM | SC | Phase 3 - 505(b)(2) NDA | TBD |
| isatuximab | Sanofi | Multiple myeloma | IV | Phase 3 - BLA; Orphan drug | TBD |
| ivosidenib | Agios | Biliary tract cancer | Oral | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| ixekizumab (Taltz®) | Eli Lilly | Axial spondyloarthritis | SC | Phase 3 - sBLA | TBD |
| lasmiditan | Eli Lilly | Migraine treatment | Oral | Phase 3 - NDA | TBD |
| lefamulin | Nabriva | CAP (bacterial) | IV, Oral | Phase 3 - NDA; Fast track; Qualified infectious diseases product | TBD |
| lemborexant | Eisai | Insomnia | Oral | Phase 3 - NDA | TBD |
| lentiviral beta-globin gene transfer | Bluebird Bio | Beta-thalissemia (transfusion-dependent) | IV | Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| lentiviral vector hematopoietic stem cell | Bluebird Bio | Cerebral adrenoleukodystrophy | N/A | Phase 3 - BLA; Breakthrough therapy; Orphan drug | TBD |
| leuprolide mesylate | Foresee | Prostate cancer | SC | Phase 3 - 505(b)(2) NDA | TBD |
| levodopa/ carbidopa (patch pump) | Mitsubishi Tanabe | Parkinson's disease motor fluctuations | SC | Phase 3 - 505(b)(2) NDA | TBD |
| levoketoconazole | Strongbridge | Cushing's syndrome | Oral | Phase 3 - NDA; Orphan drug | TBD |
| linzagolix | Obseva | Uterine fibroids | Oral | Phase 3 - NDA | TBD |
| lumateperone | Intracellular Therapies | Alzheimer's disease; Bipolar disorder | Oral | Phase 3 - NDA | TBD |
| luspatercept | Acceleron | Anemia; Myelodysplastic syndrome | SC | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| margetuximab | Macrogenics | Breast cancer | IV | Phase 3 - BLA; Fast track | TBD |
| masitinib mesylate | AB Science | Alzheimer's disease; ALS; Asthma (severe, uncontrolled); CRC; Gastrointestinal stromal tumor; Mastocytosis; MS; Melanoma; Pancreatic cancer; Prostate cancer; Ovarian cancer | Oral | Phase 3 - NDA; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| meningitis B vaccine (Trumenba®) | Pfizer | *Neisseria meningitidis* group B prevention (ages 1-9 years) | IM | Phase 3 - sBLA; Breakthrough therapy | TBD |
| mepolizumab (Nucala) | GlaxoSmithKline | Nasal polyposis; Hypereosinophilic syndrome | SC | Phase 3 - sBLA; Orphan drug | TBD |
| meropenem/ vaborbactam (Vabomere®) | The Medicines Company | HAP; Bacteremia/ septicemia; Endocarditis | IV | Phase 3 - sNDA; Qualified infectious diseases product | TBD |
| metachromatic leukodystrophy gene therapy | GlaxoSmithKline | Metachromatic leukodystrophy | IV | Phase 3 - BLA; Orphan drug | TBD |
| microbiota suspension | Rebiotix | Recurrent *Clostridium difficile* infection | Rectal | Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| midazolam spray | Upsher-Smith | Seizure disorder | Intranasal | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| minocycline | Foamix | Rosacea | Topical | Phase 3 - 505(b)(2) NDA | TBD |
| mirvetuximab soravtansine | Immunogen | Ovarian cancer | IV | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| molgramostim | Savara | Pulmonary alveolar proteinosis | Inhaled | Phase 3 - BLA; Orphan drug | TBD |
| nalbuphine ER | Trevi | Uremic pruritus | Oral | Phase 3 - NDA | TBD |
| nitric oxide | Mallinckrodt | Bronchopulmonary dysplasia | Inhaled | Phase 3 - NDA | TBD |
| nivolumab (Opdivo) | Bristol-Myers Squibb | Brain cancer; Esophageal cancer; Gastric cancer; Mesothelioma; Multiple myeloma; Ovarian cancer | IV | Phase 3 - sBLA; Orphan drug | TBD |
| nolasiban | Obseva | Reproductive disorder | Oral | Phase 3 - NDA | TBD |
| obeticholic acid (Ocaliva®) | Intercept | NASH | Oral | Phase 3 - sNDA; Breakthrough therapy | TBD |
| ofranergene obadenovec | VBL | Ovarian cancer | IV | Phase 3 - BLA | TBD |
| olaparib (Lynparza®) | AstraZeneca | Pancreatic cancer; Prostate cancer | Oral | Phase 3 - sNDA; Breakthrough therapy | TBD |
| olipudase alfa | Sanofi | Niemann-Pick disease | IV | Phase 3 - NDA; Breakthrough therapy; Orphan drug | TBD |
| omalizumab (Xolair®) | Genentech | Nasal polyposis | SC | Phase 3 - sBLA | TBD |
| ondansetron controlled-release | Redhill | Gastroenteritis | Oral | Phase 3 - 505(b)(2) NDA | TBD |
| opicapone | Neurocrine Biosciences | Parkinson's disease | Oral | Phase 3 - NDA | TBD |
| osilodrostat | Novartis | Cushing's syndrome | Oral | Phase 3 - NDA; Orphan drug | TBD |
| ozanimod | Celgene | MS; CD; UC | Oral | Phase 3 - NDA | TBD |
| pegilodecakin | ARMO | Pancreatic cancer | SC | Phase 3 - NDA; Fast track; Orphan drug | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| pegunigalsidase alfa | Protalix | Fabry disease | IV | Phase 3 - BLA; Fast track | TBD |
| pembrolizumab (Keytruda) | Merck | Breast cancer; Esophageal cancer; Pancreatic cancer; RCC; Multiple myeloma | IV | Phase 3 - sBLA; Breakthrough therapy | TBD |
| pertuzumab (Perjeta®) | Genentech | Ovarian cancer | IV | Phase 3 - sBLA | TBD |
| pexidartinib | Daiichi Sankyo | Pigmented villonodular synovitis | Oral | Phase 3 - NDA; Breakthrough therapy; Orphan drug | TBD |
| pimodivir | Janssen | Influenza treatment | Oral | Phase 3 - NDA; Fast track | TBD |
| plinabulin | Beyondspring | Neutropenia/ leukopenia; NSCLC | IV | Phase 3 - NDA | TBD |
| QPI-1002 | Quark | Delayed graft function; Kidney injury prevention following cardiac surgery | IV | Phase 3 - NDA; Orphan drug | TBD |
| quizartinib | Daiichi Sankyo | AML | Oral | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| ramucirumab (Cyramza®) | Eli Lilly | Bladder cancer; HCC | IV | Phase 3 - sBLA; Orphan drug | TBD |
| ranibizumab (biosimilar to Genentech's Lucentis®) | Santo | Wet AMD | Intraocular | Phase 3 - BLA | TBD |
| ranibizumab (Lucentis) | Genentech | Cystoid macular edema; Myopic macular degeneration; Retinopathy of prematurity | Intraocular | Phase 3 - sBLA | TBD |
| ravulizumab | Alexion | Hemolytic uremic syndrome | IV | Phase 3 - BLA; Orphan drug | TBD |
| relugolix | Myovant | Uterine fibroids; Endometriosis; Prostate cancer | Oral | Phase 3 - NDA | TBD |
| remestemcel-L | Mesoblast | Graft versus host disease | IV | Phase 3 - BLA; Fast track; Orphan drug | TBD |
| reparixin | Dompé | Transplant rejection | IV | Phase 3 - NDA; Orphan drug | TBD |
| reproxalap | Aldeyra | Congenital ichthyosis | Topical | Phase 3 - NDA; Orphan drug | TBD |
| rifabutin/ amoxicillin/ pantoprazole | Redhill | *Helicobacter pylori* infection | Oral | Phase 3 - NDA; Fast track; Qualified infectious diseases product | TBD |
| rimegepant | Portage | Migraine treatment | Oral | Phase 3 - NDA | TBD |
| risankizumab | Abbvie | PSO; CD | SC | Phase 3 - BLA; Orphan drug | TBD |
| risperidone implant | Braeburn | Schizophrenia | Implant | Phase 3 - 505(b)(2) NDA | TBD |
| rituximab (biosimilar to Genentech's Rituxan) | Amgen | RA; CLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis | IV | Phase 3 - BLA | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| rituximab (biosimilar to Genentech's Rituxan) | Pfizer | RA; CLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis | IV | Phase 3 - BLA | TBD |
| rivipansel | Pfizer | Sickle cell anemia | IV | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| rivoceranib | LSK Biopartners | Gastric cancer | Oral | Phase 3 - NDA; Orphan drug | TBD |
| ropeginterferon alfa-2b | Pharmaessentia | Polycythemia vera | SC | Phase 3 - BLA; Orphan drug | TBD |
| rovalpituzumab tesirine | Abbvie | SCLC | IV | Phase 3 - BLA; Orphan drug | TBD |
| roxadustat | AstraZeneca | Anemia due to CKD (dialysis-dependent & independent); Anemia (chemotherapy induced) | Oral | Phase 3 - NDA | TBD |
| sacubitril/ valsartan (Entresto®) | Novartis | Heart failure (preserved ejection fraction) | Oral | Phase 3 - sNDA; Fast track | TBD |
| satralizumab | Genentech | Neuromyelitis optica (Devic's syndrome) | SC | Phase 3 - BLA; Orphan drug | TBD |
| seladelpar | Cymabay | Primary biliary cirrhosis | Oral | Phase 3 - NDA; Orphan drug | TBD |
| selinexor | Karyopharm | Multiple myeloma; Sarcoma; Uterine cancer | Oral | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| selonsertib | Gilead | NASH | Oral | Phase 3 - NDA | TBD |
| selumetinib | AstraZeneca | Thyroid cancer | Oral | Phase 3 - NDA; Orphan drug | TBD |
| semaglutide | Novo Nordisk | T2DM | Oral | Phase 3 - NDA | TBD |
| seviprotimut | Polynoma | Melanoma | Intradermal | Phase 3 - BLA | TBD |
| sodium oxybate (low sodium) | Jazz | Narcolepsy | Oral | Phase 3 - NDA | TBD |
| sodium oxybate (once-nightly dosing) | Avadel | Narcolepsy | Oral | Phase 3 - 505(b)(2) NDA; Orphan drug | TBD |
| sodium thiosulfate | Fennec | Hearing loss (chemotherapy-induced) | IV | Phase 3 - 505(b)(2) NDA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| somavaratan | Versartis | Growth hormone deficiency | SC | Phase 3 - BLA; Orphan drug | TBD |
| sparsentan | Retrophin | Focal segmental glomerulosclerosis | Oral | Phase 3 - NDA; Orphan drug | TBD |
| tadalafil (versafilm) | Intelgenx | Erectile dysfunction | Oral | Phase 3 - 505(b)(2) NDA | TBD |
| tafamidis meglumine | Pfizer | Transthyretin (TTR)-related hereditary cardiomyopathy (Familial amyloid cardiomyopathy) | Oral | Phase 3 - NDA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| tasimelteon (Hetlioz®) | Vanda | Insomnia due to jet lag; Smith-Magenis syndrome | Oral | Phase 3 - sNDA | TBD |
| tecarfarin | Armetheon | Anticoagulation | Oral | Phase 3 - NDA | TBD |
| tenapanor | Ardelyx | IBS; Hyperphosphatemia | Oral | Phase 3 - NDA | TBD |

## *PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| teriparatide recombinant human (follow-on to Eli Lilly's Forteo®) | Pfenex | Osteoporosis/ osteopenia | SC | Phase 3 - 505(b)(2) NDA | TBD |
| terlipressin | Mallinckrodt | Hepatorenal syndrome | IV | Phase 3 - NDA; Fast track; Orphan drug | TBD |
| tezepelumab | AstraZeneca | Asthma (severe, uncontrolled) | SC | Phase 3 - BLA | TBD |
| timapiprant | Chiesi | Asthma | Oral | Phase 3 - NDA | TBD |
| tocilizumab (Actemra®) | Genentech | Scleroderma | SC | Phase 3 - sBLA; Breakthrough therapy | TBD |
| tralokinumab | AstraZeneca | Atopic dermatitis | SC | Phase 3 - BLA | TBD |
| treprostinil (patch pump) | Steadymed | Pulmonary arterial hypertension | SC | Phase 3 - 505(b)(2) NDA; Orphan drug | TBD |
| triamcinolone acetonide | Clearside | Uveitis | Intraocular | Phase 3 - 505(b)(2) NDA | TBD |
| trigriluzole | Portage | Obsessive compulsive disorder | Oral | Phase 3 - NDA | TBD |
| ublituximab | TG Therapeutics | CLL/SLL; MS | IV | Phase 3 - BLA; Orphan drug | TBD |
| ublituximab + umbralisib | TG Therapeutics | CLL/ SLL; DLBLC; NHL (indolent); Marginal zone lymphoma | IV + Oral | Phase 3 - NDA/BLA; Orphan drug | TBD |
| ubrogepant | Allergan | Migraine treatment | Oral | Phase 3 - NDA | TBD |
| udenafil | Allergan | Erectile dysfunction | Oral | Phase 3 - NDA | TBD |
| upadacitinib | Abbvie | RA; CD; PsA; Axial spondyloarthritis | Oral | Phase 3 - NDA | TBD |
| ursodeoxycholic acid | Retrophin | Primary biliary cholangitis | Oral | Phase 3 - NDA | TBD |
| vadadustat | Akebia | Anemia due to CKD (dialysis dependent & independent) | Oral | Phase 3 - NDA | TBD |
| valoctocogene roxaparvovec | Biomarin | Hemophilia A | IV | Phase 3 - BLA; Breakthrough therapy; Orphan drug | TBD |
| varicella-zoster vaccine (inactivated) | Merck | Herpes zoster prevention | SC | Phase 3 - BLA | TBD |
| viaskin peanut | DBV | Peanut allergy | Transdermal | Phase 3 - BLA; Breakthrough therapy; Fast track | TBD |
| vilanterol trifenatate | GlaxoSmithKline | Asthma; COPD | Inhaled | Phase 3 - NDA | TBD |
| vilaprisan | Bayer | Uterine fibroids | Oral | Phase 3 - NDA | TBD |
| vocimagene amiretrorepvec | Tocagen | Anaplastic astrocytoma; Glioblastoma | Intratumoral | Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| voclosporin | Aurinia | Lupus nephritis | Oral | Phase 3 - NDA; Fast track | TBD |
| von Willebrand factor (human, concentrate) | LFB Group | Von Willebrand disease | IV | Phase 3 - BLA; Orphan drug | TBD |

*PIPELINE DRUG LIST continued*

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| vonapanitase | Proteon | End-stage renal disease | IV | Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| vosoritide | Biomarin | Achondroplasia | SC | Phase 3 - NDA; Orphan drug | TBD |
| voxelotor | Global Blood | Sickle cell anemia | Oral | Phase 3 - NDA; Breakthrough therapy; Fast track; Orphan drug | TBD |
| VX-445 | Vertex | CF | Oral | Phase 3 - NDA; Fast track | TBD |
| VX-659 | Vertex | CF (one F508del mutation and one minimal function mutation, in combination with tezacaftor and ivacaftor) | Oral | Phase 3 - NDA | TBD |
| zolmitriptan (microneedle patch) | Zosano | Migraine treatment | Transdermal | Phase 3 - 505(b)(2) NDA | TBD |

## Complete Response Letter (CRL) / Withdrawn Drugs

| NAME | MANUFACTURER | CLINICAL USE | DOSAGE FORM | APPROVAL STATUS | FDA APPROVAL |
|---|---|---|---|---|---|
| epacadostat | Incyte | Melanoma | Oral | Withdrawn | N/A |
| furosemide pump | scPharmaceuticals | Congestive heart failure/ cardiomyopathies | SC | CRL | TBD |
| halobetasol propionate/ tazarotene | Valeant | PSO | Topical | CRL | TBD |
| insulin glargine (follow-on to Sanofi's Lantus) | Mylan/ Biocon | T1DM; T2DM | SC | CRL | TBD |
| meloxicam (nanocrystal) | Recro | Postsurgical pain | IM, IV | CRL | TBD |
| plazomicin (Zemdri™) | Achaogen | Septicemia/ bacteremia | IV | CRL | TBD |
| rituximab (biosimilar to Genentech's Rituxan) | Novartis/ Sandoz | RA; CLL/ SLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis | IV | CRL | TBD |
| rosiptor | Aquinox | Interstitial cystitis | Oral | Withdrawn | N/A |
| taselisib | Genentech | Breast cancer | Oral | Withdrawn | N/A |
| testosterone undecanoate | Lipocine | Hypogonadism | Oral | CRL | TBD |
| trastuzumab (biosimilar to Genentech's Herceptin) | Amgen | Breast cancer; Gastric/ gastroesophageal cancer | IV | CRL | TBD |
| trastuzumab (biosimilar to Genentech's Herceptin) | Pfizer | Breast cancer; Gastric/ gastroesophageal cancer | IV | CRL | TBD |

# GLOSSARY

**ABSSSI** Acute Skin and Skin Structure Infection

**ADHD** Attention Deficit Hyperactivity Disorder

**ALK** Anaplastic Lymphoma Kinase

**ALL** Acute Lymphoblastic Leukemia

**AMD** Age-Related Macular Degeneration

**AML** Acute Myeloid Leukemia

**ANDA** Abbreviated New Drug Application

**AS** Ankylosing Spondylitis

**BED** Binge Eating Disorder

**BLA** Biologics License Application

**BRCA** BReast CAncer gene

**BsUFA** Biosimilar User Fee Act

**CAP** Community Acquired Pneumonia

**CD** Crohn's Disease

**CDC** Centers for Disease Control and Prevention

**CF** Cystic Fibrosis

**CHF** Congestive Heart Failure

**CKD** Chronic Kidney Disease

**CLL** Chronic Lymphocytic Leukemia

**CMS** Centers for Medicare and Medicaid Services

**CNS** Central Nervous System

**COPD** Chronic Obstructive Pulmonary Disease

**CRC** Colorectal Cancer

**CRL** Complete Response Letter

**CV** Cardiovascular

**CVD** Cardiovascular Disease

**DEA** Drug Enforcement Administration

**DLBCL** Diffuse Large B Cell Lymphoma

**DR** Delayed-Release

**EGFR** Epidermal Growth Factor Receptor

**ER** Extended-Release

**FDA** Food and Drug Administration

**FLT3** FMS-Like Tyrosine Kinase-3

**GI** Gastrointestinal

**GLP-1** Glucagon-Like Peptide-1

**H** Half

**HAP** Healthcare-Associated Pneumonia

**HCC** Hepatocellular Carcinoma

**HCP** Healthcare Professional

**HCV** Hepatitis C Virus

**HER** Human Epidermal Growth Factor Receptor

**HER2** Human Epidermal Growth Factor Receptor 2

**HIT** Heparin Induced Thrombocytopenia

**HTN** Hypertension

**HR** Hazard Ratio

**IBS** Irritable Bowel Syndrome

**IM** Intramuscular

**IV** Intravenous

**JIA** Juvenile Idiopathic Arthritis

**LDL-C** Low-Density Lipoprotein Cholesterol

**MDD** Major Depressive Disorder

**MS** Multiple Sclerosis

**N/A** Not Applicable

**NDA** New Drug Application

**NASH** Non-Alcoholic Steatohepatitis

**NHL** Non-Hodgkin's Lymphoma

**NSAID** Non-Steroidal Anti-Inflammatory Drug

**NSCLC** Non-Small Cell Lung Cancer

**PAH** Pulmonary Arterial Hypertension

**PFS** Progression-Free Survival

**PCI** Percutaneous Coronary Intervention

**PDUFA** Prescription Drug User Fee Application

**PsA** Psoriatic Arthritis

**PSO** Plaque Psoriasis

**PTCA** Percutaneous Transluminal Coronary Angioplasty

**Q** Quarter

**QOL** Quality of Life

**RA** Rheumatoid Arthritis

**RCC** Renal Cell Carcinoma

**SL** Sublingual

**sBLA** supplemental Biologics License Application

**SC** Subcutaneous

**SCCHN** Squamous Cell Cancer of the Head and Neck

**SCLC** Small Cell Lung Cancer

**SLE** Systemic Lupus Erythematosus

**SLL** Small Lymphocytic Lymphoma

**sNDA** supplemental New Drug Application

**SR** Sustained-Release

**SSSI** Skin and Skin Structure Infection

**T1DM** Type 1 Diabetes Mellitus

**T2DM** Type 2 Diabetes Mellitus

**TBD** To Be Determined

**UA** Unstable Angina

**UC** Ulcerative Colitis

**US** United States

**UTI** Urinary Tract Infection

**WHO** World Health Organization

**XR** Extended-Release

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