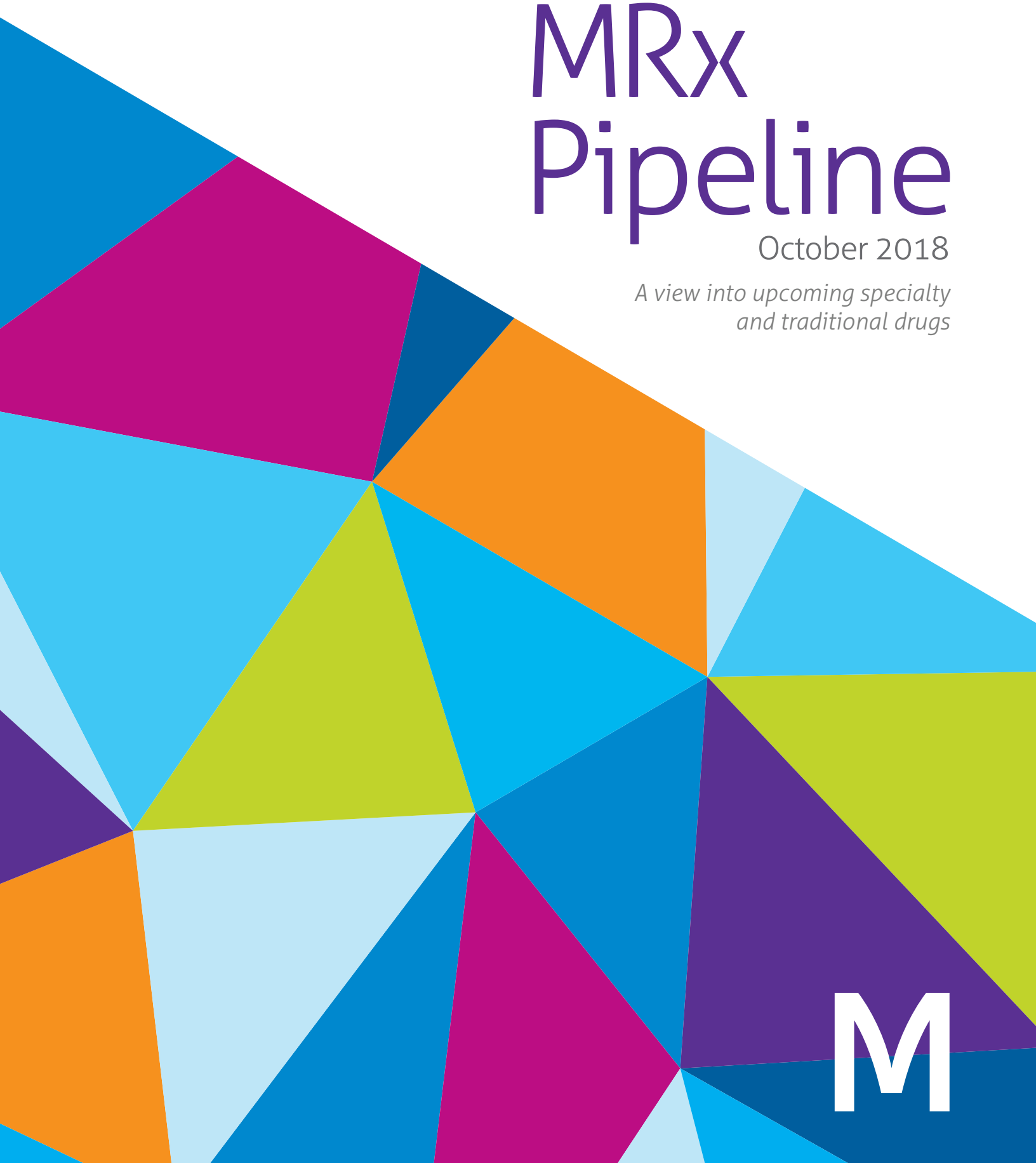
Magellan Rx MANAGEMENT℠

MRx Pipeline

October 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.

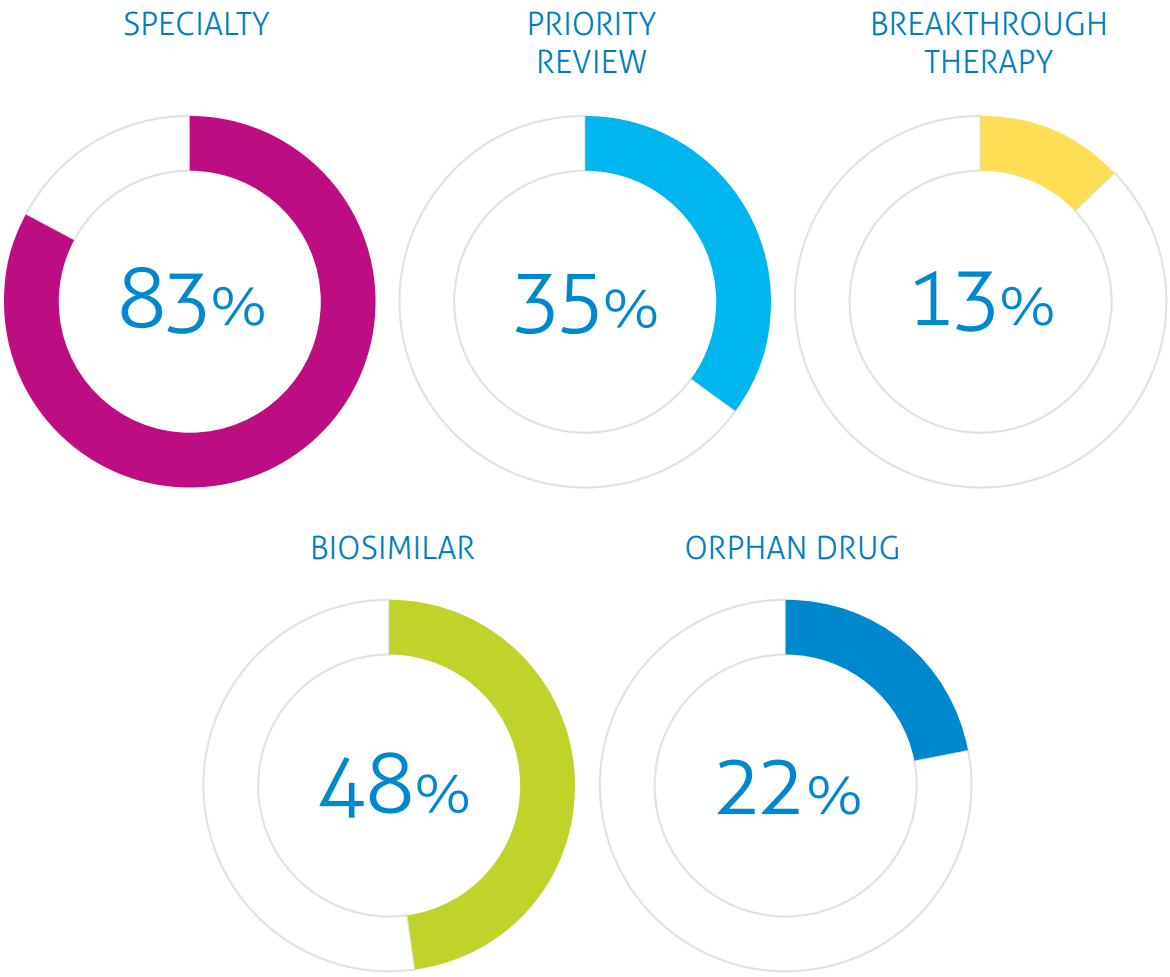
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year 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.

Last year marked a 21-year record high number of approvals as the FDA granted approvals to 46 novel drugs. Thus far in 2018, the FDA has approved 47 novel drugs, already surpassing 2017 approvals. 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 including first-time approvals for select biosimilars, digital therapeutics, and new treatment modalities using gene therapy is expected. Noteworthy pipeline trends to watch in the upcoming quarters include the development of complex therapies, therapeutic options for rare hereditary diseases, oncology, immunology, and neurology. Moreover, investigational products for Alzheimer's disease, hemophilia, multiple sclerosis, women's health, infectious diseases, and peanut allergies, 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.

brexanolone *IV*

Sage

PROPOSED INDICATIONS

Treatment of postpartum depression (PPD)

CLINICAL OVERVIEW

Brexanolone is an allosteric $GABA_A$ receptor modulator. Preclinical studies have shown that typically $GABA_A$ levels decrease during pregnancy and then increase after birth. However, if regulation of these levels is impaired, depression may occur.

Two separate, double-blind, Hummingbird trials evaluated brexanolone, administered as a 1-time, 60-hour continuous IV infusion, in adult women who were ≤ 6 months postpartum and experiencing moderate (n=104) or severe (n=122) PPD beginning in the third trimester of pregnancy or within 4 weeks of delivery. Women either ceased lactating or agreed to temporarily stop breastfeeding during the study. At 60 hours, women with severe PPD (HAM-D ≥ 26 points) experienced significant mean reductions in HAM-D with brexanolone 60 and 90 µg/kg/hr compared to placebo (19.9 and 17.7 versus 14 points, respectively). Similarly, in women with moderate PPD (HAM-D of 20 to 25 points), mean reductions in HAM-D of 14.2 points with brexanolone 90 µg/kg/hr and 12 points with placebo were reported. Statistically significant differences from placebo were seen as early as 24 hours for both doses. While the effect reported at 60 hours was maintained through day 30 in women with moderate and severe PPD, the difference compared to placebo was only statistically significant in women with severe PPD. Brexanolone was well tolerated. Common adverse effects were headache, dizziness, and somnolence. Two patients on brexanolone experienced a serious reaction: 1 patient experienced suicidal ideation and intentional overdose attempt during follow-up and 1 patient reported an altered state of consciousness and syncope.

PLACE IN THERAPY

An estimated 14% to 23% of pregnant women experience depression during pregnancy; 5% to 25% experience depression postpartum. The American College of Obstetricians and Gynecologists (ACOG) advise screening for depression in the perinatal period, particularly during the "fourth trimester" of pregnancy—the stressful weeks to months following delivery when a woman is recovering from childbirth, affected by changing hormones, and learning to care for her newborn child.

Psychotherapy is suggested to treat mild to moderate PPD, while the addition of antidepressant therapy is recommended for severe cases. Antidepressant options include selective serotonin reuptake inhibitors (SSRIs), serotonin-norepinephrine reuptake inhibitors (SNRIs), bupropion, and mirtazapine.

Brexanolone has demonstrated safety and effectiveness for moderate and severe PPD. If approved, it will be the first medication indicated specifically for PPD and is associated with significant improvement in depressive symptoms within 24 to 60 hours compared to 2 to 6 weeks with current antidepressant medications. In clinical trials, breastfeeding was interrupted for 7 days and the 60-hour infusion was administered in a clinical setting, both of which may hinder market uptake of the product. It remains to be seen if the infusion will be given on an outpatient basis. Sage Therapeutics is investigating a daily oral option as well as safety and efficacy of the infusion in treating PPD in females aged 15 to 17 years.

FDA APPROVAL TIMELINE

December 19, 2018

✓ Breakthrough Therapy ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$34	$99	$139	$189

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

Immunology

caplacizumab *IV*

Sanofi

PROPOSED INDICATIONS

Treatment of acquired thrombotic thrombocytopenic purpura (aTTP) in adults

CLINICAL OVERVIEW

Caplacizumab is a bivalent nanobody von Willebrand factor inhibitor that prevents platelet adhesion.

HERCULES, a phase 3, double-blind trial, evaluated caplacizumab as add-on to standard of care (daily plasma exchange [PEX] and immunosuppression) in adults (n=145) with aTTP who require daily PEX. The study demonstrated a statistically significant reduction with caplacizumab compared to placebo in time to platelet count response ($p<0.01$); patients treated with caplacizumab were 1.5 times more likely to achieve platelet count response than those treated with placebo. During the treatment period, caplacizumab led to a 74% reduction in composite incidence of aTTP-related death, aTTP recurrence, or a major thromboembolic event. This was primarily due to a reduction in aTTP recurrence. During treatment, no deaths occurred in the caplacizumab group, while 3 deaths (4.1%) were reported in the placebo group. There was no difference in incidence of major thromboembolic events. In addition, a trend toward faster normalization of organ damage markers (e.g., lactate dehydrogenase, cardiac troponin I, serum creatinine) was reported with caplacizumab. Bleeding-related adverse events occurred more often with caplacizumab than placebo (66.2% versus 49.3%); however, most were of mild to moderate severity. Rates of refractory aTTP were low in both groups.

In the study, caplacizumab was initiated as a 10 mg IV bolus dose followed by 10 mg SC daily until 30 days after the last daily PEX. If evidence of underlying disease activity persisted, treatment was extended up to another 28 days.

PLACE IN THERAPY

Typically diagnosed in adults, aTTP is an autoimmune disorder. It is characterized by formation of blood clots in small blood vessels causing thrombocytopenia, hemolytic anemia, neurologic deterioration, and organ damage. An estimated 1.7 to 11 million people in the US are affected by thrombotic thrombocytopenic purpura each year, with 95% of cases due to aTTP (5% due to the genetic form). Episodes have a sudden onset, and if not treated promptly, can lead to death. Recurrent episodes occur in up to 60% of patients with aTTP and usually last for days or weeks but may continue for months. The standard of care (SOC) is daily PEX, which has a success rate of over 80%; it is typically given in combination with corticosteroids and off-label rituximab. Caplacizumab, as add-on to SOC, has proven to reduce aTTP recurrence and related death. There are currently no other products in late-stage development for aTTP.

FDA APPROVAL TIMELINE

February 6, 2019

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$30	$78	$134	$190

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

Neurology

cladribine *oral*

Merck

PROPOSED INDICATIONS

Treatment of relapsing forms of multiple sclerosis (MS)

CLINICAL OVERVIEW

Cladribine is a purine nucleoside analogue that targets lymphocytes, which are involved in the pathogenesis of MS lesions.

The phase 3, CLARITY trial assessed the effect of cladribine compared to placebo in patients (n=1,326) with relapsing remitting MS (RRMS). At 96 weeks, the annualized qualifying relapse rate (ARR) was significantly lower with cladribine, given as cumulative yearly doses of 3.5 or 5.25 mg/kg, compared to placebo (ARR, 0.14, 0.15, and 0.33, respectively); this translated to a relative reduction for each dose compared to placebo of 57.6% and 54.5%, respectively. Lymphopenia was the most common adverse event reported in patients treated with cladribine (21.4% and 31.3% with doses of 3.5 and 5.25 mg/kg, respectively). The 2-year extension trial showed sustained response in cladribine-treated patients, including those with high disease activity; *post hoc* analyses showed a sustained status of no evidence of disease activity (NEDA) through year 4.

In CLARITY, cladribine was administered as a cumulative dose of 0.875 mg/kg over 5 consecutive days of a 28-day period. The dose was given during weeks 1, 5, 9, 13, 48, and 52, resulting in a total dose of 5.25 mg/kg, or during weeks 1, 5, 48, and 52 (matching placebo at weeks 9 and 13), resulting in a total dose of 3.5 mg/kg.

PLACE IN THERAPY

Of the almost 1 million people in the US living with MS, the majority have RRMS. While there are several disease modifying therapies available that are effective in treating RRMS, cladribine offers a unique mechanism of action and is the only oral formulation that may require as few as 20 doses in the course of 2 years, with possibly no further re-treatment required. Notably, the FDA and the European Medicines Agency (EMA) refused to approve cladribine in 2011 based on concerns of malignancy risks. Since that time, new, long-term safety data demonstrated no increased cancer risk with the medication. Subsequently, in August 2017, the EMA approved cladribine for highly-active relapsing MS.

FDA APPROVAL TIMELINE

January 30, 2019

✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$10	$19	$32	$46

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

Oncology

gilteritinib *oral*

Astellas

PROPOSED INDICATIONS

Treatment of relapsed or refractory acute myeloid leukemia (R/R AML) with an FLT3 mutation

CLINICAL OVERVIEW

Gilteritinib is a second-generation tyrosine kinase inhibitor (TKI) with activity against FLT3, ALK, and AXL receptor tyrosine kinase.

A phase 1/2, open-label, dose escalation (n=23) and dose expansion (n=229) study evaluated gilteritinib at doses ranging from 20 mg to 450 mg once daily in patients with R/R AML. Overall response rate (ORR) of 40% (including 8% complete response) was recorded among all dosages regardless of presence of FLT3 mutation. Higher ORR was seen with daily gilteritinib doses ≥ 80 mg in patients with FLT3 or FLT3-internal tandem duplication (ITD) mutations (ORR, 52% and 55%, respectively). In addition, a lower ORR of 37% was reported in patients who received prior TKI therapy. Among all gilteritinib doses, the median overall survival (OS) was 25 weeks, and a longer median OS of 31 weeks was seen in FLT3+ patients. QTcF prolongation (> 500 ms) occurred in 4% of patients. The most common grade 4 adverse events included febrile neutropenia (39%), anemia (24%), thrombocytopenia (13%), sepsis (11%), and pneumonia (11%). A maximally tolerated dose of 300 mg was established.

Data from the phase 3, open-label, ADMIRAL trial were submitted to the FDA to support approval of gilteritinib. The ongoing unpublished study compares gilteritinib 120 mg per day with salvage chemotherapy in patients (n=369) with R/R AML and FLT3 mutation.

PLACE IN THERAPY

AML is the most common acute leukemia in adults with an estimated 19,520 new cases and 10,670 deaths expected in the US in 2018. Median age of onset is 67 years. AML is associated with low survival rates, particularly in patients with FLT3 mutations—a population with unmet treatment need which accounts for about a third of all cases. Treatment for R/R AML includes cytotoxic regimens.

If approved, gilteritinib will be the second FLT3 inhibitor approved in the AML space, following first-generation oral midostaurin (Rydapt®) that has not proven itself for R/R AML. While gilteritinib is seeking an indication for R/R AML, midostaurin is approved for first-line treatment combined with chemotherapy in newly diagnosed patients. Moreover, gilteritinib also targets AXL, which may bypass resistance mechanisms to FLT3 inhibitors. Other late-stage investigational agents for FLT3+ AML include oral crenolanib and quizartinib. Gilteritinib is also being studied in other AML populations including treatment-naïve FLT3+ patients (high-intensity chemotherapy ineligible), where it could compete with midostaurin, and in patients with FLT3-ITD mutation who are in complete remission after standard induction chemotherapy and allogeneic SCT. An FLT3 companion diagnostic test is also being reviewed for approval.

FDA APPROVAL TIMELINE

November 29, 2018

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$3	$34	$59	$101	$134

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

Oncology

glasdegib *oral*

Pfizer

PROPOSED INDICATIONS

Treatment of acute myeloid leukemia (AML) in combination with low-dose cytarabine (LDAC) in treatment-naïve adults

CLINICAL OVERVIEW

Glasdegib inhibits the smoothened (SMO) receptor, a membrane protein that plays a role in the Hedgehog pathway. Abnormal activation of the pathway can lead to hematologic malignancies and solid tumors.

In the phase 2, open-label, BRIGHT 1003 study (n=132), the addition of glasdegib to LDAC reduced the risk of death by nearly half versus LDAC alone in treatment-naïve patients with AML or high-risk myelodysplastic syndrome who were not candidates for intensive chemotherapy. The median OS was 8.8 versus 4.9 months, respectively (HR, 0.5; 95% CI, 0.33 to 0.75; p=0.0003). While febrile neutropenia was reported more often with glasdegib (36.9% versus 26.8% with LDAC), septicemia was reported less often when glasdegib was added (3.6% versus 12.2%, respectively). Additionally, patients who received glasdegib experienced increased taste distortion (23.8%), muscle spasms (20.2%), and thinning or loss of hair (10.2%).

The ongoing, phase 3, BRIGHT AML 1019 trial is exploring glasdegib combined with intensive (cytarabine ± daunorubicin chemotherapy or hematopoietic SCT) or nonintensive (azacitidine) therapies in treatment-naïve AML patients. Results have not been published.

Glasdegib was studied as 100 mg orally once daily in 28-day cycles on a continuous basis. LDAC was administered as 20 mg SC twice daily on days 1 through 10 of each cycle.

PLACE IN THERAPY

AML is an aggressive, difficult to treat, hematopoietic malignancy associated with high mortality. It is the most common acute leukemia in adults with an estimated 19,520 new cases and 10,670 deaths expected in the US in 2018. Median age of onset is 67 years. It is associated with a poor prognosis, particularly in patients who are ineligible for intensive chemotherapy. Glasdegib is the first SMO inhibitor that, if approved, could provide benefit to AML patients who have few treatment options.

FDA APPROVAL TIMELINE

December 2018

✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$41	$112	$186	$243

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

Immunology

ravulizumab *IV, SC*

Alexion

PROPOSED INDICATIONS

Treatment of paroxysmal nocturnal hemoglobinuria (PNH)

CLINICAL OVERVIEW

Ravulizumab is a long-acting C5 complement inhibitor. It is the next-generation of eculizumab (Soliris®; Alexion).

In a 26-week, open-label trial, ravulizumab was determined to be noninferior to eculizumab for the treatment of PNH in treatment-naïve adults (n=246). This was based on results showing a higher proportion of patients treated with ravulizumab remained transfusion-free (73.6% versus 66.1%, respectively), and achieved lactate dehydrogenase (LDH) normalization (53.6% versus 49.4%, respectively) compared to those treated with eculizumab. The most common treatment-related adverse effect with ravulizumab was headache. Major CV events were reported in 2 patients treated with ravulizumab and 1 with eculizumab. No meningococcal infections were reported. A second, open-label study demonstrated non-inferiority when patients (n=195) were switched from stable doses of eculizumab to ravulizumab. Patients also reported a significant improvement in QOL as measured by the functional assessment of chronic illness therapy (FACIT)-fatigue scale. Ravulizumab was generally well tolerated, with a safety profile similar to eculizumab. Headache was reported most often; pyrexia was the most serious adverse event.

In both studies, weight-based doses of ravulizumab were administered IV as a loading dose, and every 8 weeks thereafter. The eculizumab dosage was consistent with its approved label.

PLACE IN THERAPY

PNH is a rare blood disorder occurring in about 0.5 to 1.5 per million people worldwide. Diagnosis is typically made during the fourth decade of life. In PNH, the bone marrow produces abnormal red blood cells (RBCs) that prematurely hemolyze, resulting in hemolytic anemia and chronic hemoglobinuria, which can worsen during illness, trauma, or stress. Most patients experience fatigue and difficulty breathing. Individuals with PNH are susceptible to developing potentially life-threatening blood clots. Allogeneic hematopoietic cell transplant is the only curative therapy for PNH.

Eculizumab, which is the only drug available in the US to manage PNH, can increase a patients' risk of meningococcal infections. Ravulizumab has been shown to be non-inferior to eculizumab. While eculizumab is dosed once every 2 weeks, ravulizumab is dosed once every 8 weeks, thereby providing a more desirable maintenance dosing schedule. If approved, patients currently treated with eculizumab will most likely switch to ravulizumab prior to the anticipated 2021 approval of eculizumab biosimilars. Ravulizumab is in phase 3 trials for atypical hemolytic uremic syndrome (aHUS) and preclinical investigation for myasthenia gravis (gMG), indications that are held by eculizumab. A ravulizumab SC formulation is also being studied.

FDA APPROVAL TIMELINE

February 18, 2019

✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$3	$10	$23	$43

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

Immunology

risankizumab *SC*

Abbvie/Boehringer Ingelheim

PROPOSED INDICATIONS

Treatment of moderate to severe plaque psoriasis (PSO)

CLINICAL OVERVIEW

Risankizumab is an interleukin-23 (IL-23) inhibitor.

The identical, double-blind, double-dummy ultIMMa-1 and ultIMMa-2 trials (n=797 total) demonstrated significantly greater improvement in moderate to severe PSO with risankizumab compared to a European-sourced ustekinumab (Stelara®), as measured by the proportion of patients who achieved PASI 90 and a sPGA score of clear (0) or almost clear (1). At week 16, in ultIMMa-1, respective scores for risankizumab and ustekinumab for PASI 90 were 75.3% and 42%, and sPGA were 87.8% and 63%. In ultIMMa-2, respective scores for risankizumab and ustekinumab for PASI 90 were 74.5% and 47.52%, and sPGA were 83.7% and 61.6%. These rates were maintained at week 52.

Similarly, in the phase 3 IMMvent study (n=605), at week 16, PASI 90 and sPGA = 0/1 were achieved in 72% and 84% of patients treated with risankizumab, respectively, compared to 47% and 60%, respectively, with adalimumab (Humira®). The study also demonstrated benefit with risankizumab in patients who failed adalimumab (PASI < 50%); PASI 90 was 66% with risankizumab versus 21% with adalimumab.

In the clinical trials, risankizumab was given as a flat dose of 150 mg; ustekinumab dose was weight-based as 45 mg or 90 mg. Both medications were administered SC at weeks 0, 4, 16, 28, and 40. The initial dose of adalimumab was 80 mg SC, followed by 40 mg SC every other week starting 1 week after the initial dose.

PLACE IN THERAPY

An estimated 7.5 million Americans are living with PSO with 20% of cases considered moderate to severe. The 2008 American Academy of Dermatology (AAD) guidelines consider the TNFα inhibitors, adalimumab (Humira), etanercept (Enbrel®), and infliximab (Remicade®), and the IL-12/23 inhibitor, ustekinumab (Stelara), as acceptable options for PSO after failure of topical therapy alone when phototherapy is not available; however, many of the newer agents were not available at the time of guideline development.

Risankizumab demonstrated significant improvement in PSO severity over the PSO market leaders adalimumab and ustekinumab. Although studied as a first-line biologic option, given the current treatment landscape and provider feedback, it will likely compete directly against IL-23 inhibitors guselkumab (Tremfya®) and tildrakizumab-asmn (Ilumya™), as well as other IL inhibitors, such as ustekinumab, secukinumab (IL-17A inhibitor; Cosentyx®), and ixekizumab (IL-17A inhibitor; Taltz®) after failure of the well-established TNFα inhibitors, like adalimumab. Emergence of TNFα inhibitor biosimilars will also impact market uptake of risankizumab; biosimilars infliximab-abad (Renflexis™) and infliximab-dyyb (Inflectra®) are currently available in the US. Risankizumab is also in phase 3 trials for Crohn's disease and ulcerative colitis.

FDA APPROVAL TIMELINE

April 25, 2019

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$111	$238	$407	$612

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

Oncology

sacituzumab govitecan *IV*

Immunomedics

PROPOSED INDICATIONS

Treatment of metastatic triple-negative breast cancer (mTNBC) following ≥ 2 prior therapies

CLINICAL OVERVIEW

Sacituzumab govitecan is part of an emerging class of anticancer treatments called antibody drug conjugates (ADC), which combines a monoclonal antibody with cytotoxic chemotherapy. This specific product consists of a humanized immunoglobulin (IgG) monoclonal antibody linked with the active metabolite of irinotecan, SN-38. The combination targets Trop-2, a trophoblastic cell-surface antigen that is highly expressed in over 90% of triple-negative breast cancer (TNBC) cells. Furthermore, ADCs are designed to identify and kill the cancer cell while minimizing systemic toxicity.

In a single-arm, phase 2 trial, sacituzumab govitecan produced an objective response rate (ORR) of 34% in patients (n=110) with at least 2 prior treatments for mTNBC. Complete response was experienced by 3 patients and partial response was experienced by 34 patients. Median duration of response was 7.6 months, median PFS was 5.5 months, and median OS was 12.7 months. Grade 3/4 adverse events included neutropenia (41%), leukopenia (14%), diarrhea (8%), and febrile neutropenia (7%).

Sacituzumab govitecan 10 mg/kg was administered IV on days 1 and 8 of a 21-day cycle; cycles were repeated until progression or unacceptable toxicity. A median number of 14.5 doses (range, 1 to 88) was administered in the study.

PLACE IN THERAPY

About 12% of women in the US will develop invasive breast cancer during their lifetime, while lifetime risk in men is about 0.1%. TNBC accounts for 10% to 20% of all breast cancers and occurs twice as often in African American women than Caucasian women. TNBC refers to breast cancers that do not express genes for estrogen receptor (ER), progesterone receptor (PR), and human epidermal growth factor receptor 2 (HER2); therefore, TNBC does not respond to hormonal therapy, such as tamoxifen or aromatase inhibitors, or therapies targeting HER2 receptors, such as trastuzumab (Herceptin®). TNBC has a poorer short-term prognosis than other breast cancer subtypes, partly due to the lack of targeted therapies. Currently, no FDA-approved agent is indicated specifically to treat TNBC.

If approved, sacituzumab govitecan will be the first ADC therapy targeting Trop-2 and will represent a new alternative to traditional chemotherapy for pretreated TNBC. Uptake is expected to be modest due to the small population size and short duration of treatment in this population. Other products in late-stage development for TNBC include PD-1 inhibitors, atezolizumab (Tecentriq®) and pembrolizumab (Keytruda®), and PARP inhibitors, niraparib (Zejula®), talazoparib (Talzenna™), and investigational veliparib. The investigational ADC, glembatumumab vedotin, failed to show a durable response against mTNBC.

FDA APPROVAL TIMELINE

January 18, 2019

✓ Breakthrough Therapy ✓ Fast Track ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$1	$2	$3	$5

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

Behavioral Health

samidorphan/buprenorphine *SL*

Alkermes

PROPOSED INDICATIONS

Adjunctive treatment of major depressive disorder (MDD) in patients with inadequate response to standard antidepressant therapies

CLINICAL OVERVIEW

Samidorphan is a selective mu opioid full antagonist that does not effect kappa and delta opioid receptors. Buprenorphine is a partial mu agonist and kappa-receptor antagonist. Kappa antagonism of the fixed-dose combination may provide antidepressant activity, without mu opioid agonism that increases abuse potential.

The phase 3, double-blind, FORWARD-5 clinical trial demonstrated a significant improvement in depression symptoms with samidorphan/buprenorphine 2 mg/2 mg once daily as adjunct to standard antidepressant therapy in patients (n=407) with MDD who had an inadequate response to antidepressant therapy. Samidorphan/buprenorphine resulted in statistically significant improvements over placebo in the average change in the MADRS-6 and MADRS-10 scores from week 3 to end of treatment at 11 weeks. While the 1 mg/1 mg daily dose did not lead to statistically significant changes in FORWARD-5, when data were combined with the similarly designed FORWARD-4 study, statistical significance was reported. Further, in the 12-month, open-label, FORWARD-2 extension trial (n=1,454), of the 49% of patients who completed the study, 52.5% achieved remission; the median time to remission was 59 days. Common side effects included nausea, headache, constipation, dizziness, and somnolence. Withdrawal was not evident upon stopping therapy.

PLACE IN THERAPY

In 2016, about 16.2 million (6.7%) adults in the US experienced at least 1 major depressive episode, leading to severe impairment in 10.3 million (4.3%) of cases. First-line treatment of MDD includes an SSRI, SNRI, bupropion, or mirtazapine. Select atypical antipsychotics may be added when an adequate response is not achieved after multiple trials with antidepressant therapy alone. If approved, once-daily samidorphan/buprenorphine may compete with antipsychotics in the adjunct setting and lack metabolic disturbances (e.g., weight gain, elevated glucose and lipids) associated with antipsychotics. While abuse potential of samidorphan/buprenorphine appears to be low, a potential Schedule III controlled substance designation could impact its market uptake. Investigational esketamine (Janssen) and rapastinel (Allergan), both which target glutamate, are in phase 3 trials for MDD. Alkermes is also studying samidorphan in combination with olanzapine to treat schizophrenia and bipolar disorder.

FDA APPROVAL TIMELINE

January 31, 2019

✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$13	$42	$78	$128

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

Neurology

siponimod *oral*

Novartis

PROPOSED INDICATIONS

Treatment of secondary progressive multiple sclerosis (SPMS)

CLINICAL OVERVIEW

Siponimod is a selective sphingosine-1-phosphate receptor-1 and -5 modulator that may reduce inflammation and cell damage, and inhibit neurologic deficits in patients with MS.

Safety and efficacy of siponimod were studied in the 60-month, phase 3, EXPAND trial, which included 1,651 adults (aged 18 to 60 years) with SPMS and a baseline EDSS score of 3 to 6.5. The study demonstrated a 21% reduction in risk of disability progression with siponimod over placebo at 3 months and 26% at 6 months. Siponimod treatment slowed brain volume loss by 23% and limited the increase in brain lesion volume compared to placebo. It also reduced the ARR by 55%. At 24 months, siponimod improved cognitive processing speed compared to placebo; however, it had no effect on the timed 25-foot walk test or memory. Serious adverse events occurred in 18% of patients treated with siponimod versus 15% with placebo. Bradycardia, bradyarrhythmia, hypertension, lymphopenia, liver transaminase elevations, macular edema, seizures, and varicella zoster reactivation were reported more often with siponimod than placebo. An initial dose titration mitigated cardiac effects.

Siponimod doses were titrated from 0.25 mg to 2 mg orally once daily.

PLACE IN THERAPY

It is estimated that nearly 1 million people in the US are living with MS. The majority have RRMS, and while the disease course is highly variable, patients can eventually progress to SPMS, experiencing a gradual worsening of neurological function. Evidence suggests that during SPMS there is an expansion of existing lesions, rather than an increase in new active lesions as with RRMS. Cognitive decline is also more severe.

Currently, there are several disease-modifying therapies approved by the FDA for RRMS, and one (ocrelizumab [Ocrevus®]) is approved to treat primary progressive MS; however, none of these have proven to be effective for SPMS. If approved, siponimod will be the first medication to treat SPMS and may also provide a positive effect on cognitive function. Siponimod is designed to be a more selective agent compared to the sphingosine 1-phosphate receptor modulator fingolimod (Gilenya®), also by Novartis, but the impact of this selectivity has not been established. Unlike fingolimod and some other MS agents, to date, siponimod has not been associated with progressive multifocal leukoencephalopathy (PML), a rare and life-threatening viral brain infection.

FDA APPROVAL TIMELINE

March 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$83	$220	$413	$562

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

Endocrine

sotagliflozin *oral*

Sanofi

PROPOSED INDICATIONS

Treatment of type 1 diabetes mellitus (T1DM)

CLINICAL OVERVIEW

Sotagliflozin is a sodium-glucose cotransporter-1 and -2 (SGLT1/SLGT2) inhibitor. While SGLT1 inhibition primarily prevents resorption of glucose and galactose in the GI tract, inhibition of SGLT2 blocks resorption of glucose in the kidney.

The phase 3, double-blind, inTandem1 study evaluated sotagliflozin in 793 patients with T1DM not adequately controlled with insulin therapy. Daily doses of sotagliflozin 200 mg or 400 mg, as add-on to optimal insulin treatment, produced significant placebo-adjusted HbA1c reductions of 0.36% and 0.41%, respectively, at 24 weeks and 0.25% and 0.31%, respectively, at 52 weeks. At 24 weeks, HbA1c < 7% was achieved by 15.7%, 27.2%, and 40.3% of patients receiving placebo, sotagliflozin 200 mg, and sotagliflozin 400 mg, respectively. At 52 weeks, significant placebo-adjusted decreases in fasting plasma glucose (19.5 mg/dL), weight (4.3 kg), bolus insulin dose (15.6%), and basal insulin dose (11.9%) were seen with sotagliflozin 400 mg. In each sotagliflozin group, 6.5% of patients experienced severe hypoglycemia compared to 9.7% in the placebo group. Adverse events that occurred more frequently with sotagliflozin compared to placebo were diabetic ketoacidosis, genital mycotic infections, and diarrhea. Similar findings were reported in the inTandem3 clinical trial.

PLACE IN THERAPY

In the US, approximately 23 million people are diagnosed with diabetes, of which about 5% have T1DM. Several SGLT2 inhibitors are currently FDA-approved to treat T2DM as single drug entities or as fixed-dose combinations with metformin or a DPP-4 inhibitor. If approved, sotagliflozin will be the first antidiabetic agent that blocks both SGLT1 and SGLT2, thereby, acting in both the GI tract and the kidneys. It will also be the first oral medication to be used in combination with insulin for T1DM. Sotagliflozin is also in late-stage development for the treatment of T2DM.

FDA APPROVAL TIMELINE

March 22, 2019

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$71	$126	$256	$436

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

Oncology

tagraxofusp *IV*

Stemline

PROPOSED INDICATIONS

Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN)

CLINICAL OVERVIEW

BPDCN is a rare, aggressive hematologic malignancy that displays features of both leukemia and lymphoma. Cutaneous lesions are characteristic in 80% of cases.

Tagraxofusp is a protein consisting of human interleukin-3 (IL-3) fused to truncated diphtheria toxin. It is designed to target the IL-3 receptor (CD123), which is highly expressed on some cancer stem cells.

In a pivotal, phase 1/2, single-arm trial, treatment with tagraxofusp resulted in a 90% overall response rate (ORR) in treatment-naïve patients (12 µg/kg; n=29). Complete response (CR), including clinical complete response (CRc) and complete response with incomplete hematologic recovery (CRi), occurred in 72% of patients, and partial response (PR) occurred in 17%. Tagraxofusp was a bridge to SCT in 45% of the patients. In addition, patients with relapsed or refractory BPDCN, achieved an ORR of 69% with tagraxofusp (n=13); CR+CRC+Cri was reported in 38% of patients, PR in 31%, and 8% of patients were bridged to SCT. Median follow-up in this study was 13.8 months, at which time OS had not been reached in treatment-naïve patients; historical median OS is 8 to 14 months. The most commonly reported treatment-related adverse events were liver transaminase elevations (52%), hypoalbuminemia (50%), and thrombocytopenia (38%).

Tagraxofusp was administered IV over 15 minutes once daily for the first 5 consecutive days of a 21-day cycle, for up to 6 cycles. Increasing doses were used for each new cohort of patients to receive tagraxofusp as defined in the study design. In stage 1, tagraxofusp dosage was 7, 9, 12, or 16 µg/kg/day. In stages 2 and 3, the dose was 12 µg/kg/day. Enrollment for stage 4 is ongoing.

PLACE IN THERAPY

BPDCN most commonly occurs in the elderly (aged 60 to 70 years), yet can be seen in children as well. It accounts for < 1% of all hematologic malignancies but is often misdiagnosed as NHL, AML, leukemia cutis, melanoma, or lupus erythematosus. There is no established treatment for BPDCN. Current first-line treatment includes chemotherapy regimens utilized for ALL. Allogeneic SCT is recommended in patients who relapse, which usually occurs within 2 years of remission.

Tagraxofusp appears to have significant activity against this extremely rare condition, particularly in treatment-naïve patients. If approved, it will be the first available agent that targets CD123 and the only drug indicated to treat BPDCN. Its place in therapy remains to be seen. It is possible for it to be used with induction/consolidation chemotherapy to achieve remission in SCT candidates, particularly in the relapsed/refractory setting. It also has potential as monotherapy or in combination with low-dose chemotherapy. Tagraxofusp is also being studied for several other hematologic malignancies.

FDA APPROVAL TIMELINE

February 21, 2019

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$0	$27	$55	$116	$163

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 the 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 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 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 6 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) 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. Subsequently, 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.

Biosimilars 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 *SC*

Hyrimoz and SB5 are biosimilars 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

Novartis/Sandoz (Hyrimoz)
November 16, 2018

Samsung Bioepis/Merck (SB5)
May 2019

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$13,718	$15,080	$16,156	$17,125	$17,743

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

Blood Modifier
filgrastim (Grastofil) *IV, SC*

Adello, Apotex, and Tanvex 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
Pending

Apotex (Grastofil)
Pending

Tanvex (TX01)
August 1, 2019

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$246	$207	$186	$170	$157

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

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$2,053	$1,625	$1,286	$1,024	$878

The forecast is a projection of total US sales per year for the *branded originator 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
$3,739	$3,178	$2,704	$2,284	$1,914

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

** On October 10, 2018, an FDA advisory committee recommended approval of Truxima for 3 NHL indications.*

FDA APPROVAL TIMELINE
November 30, 2018

FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$3,984	$3,179	$2,482	$2,010	$1,644

The forecast is a projection of total US sales per year for the *branded originator 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)
Pending

Teva/Celltrion (Herzuma)
December 18, 2018

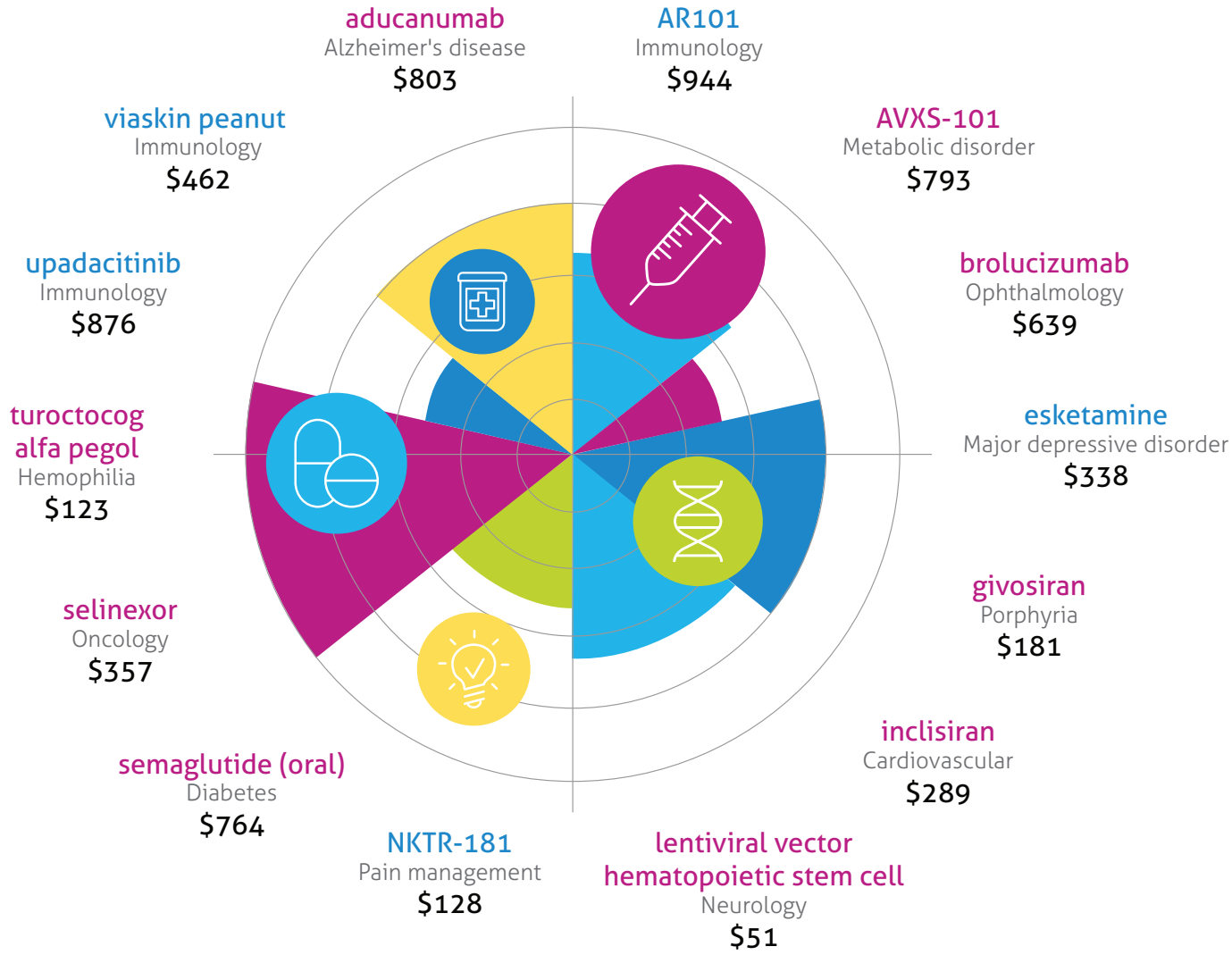
FINANCIAL FORECAST (reported in millions)

2018	2019	2020	2021	2022
$2,816	$2,392	$1,812	$1,406	$1,120

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

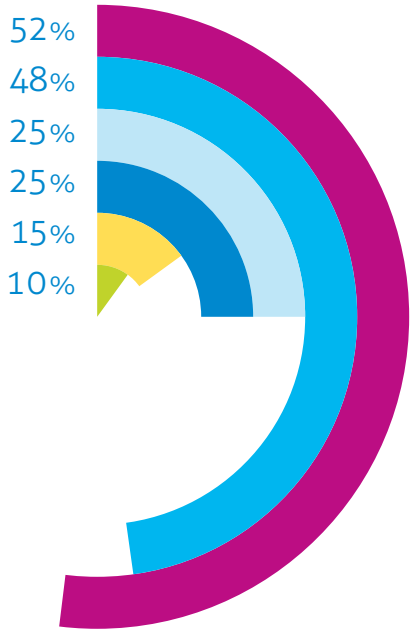


★ Specialty drug names appear in magenta throughout the publication.

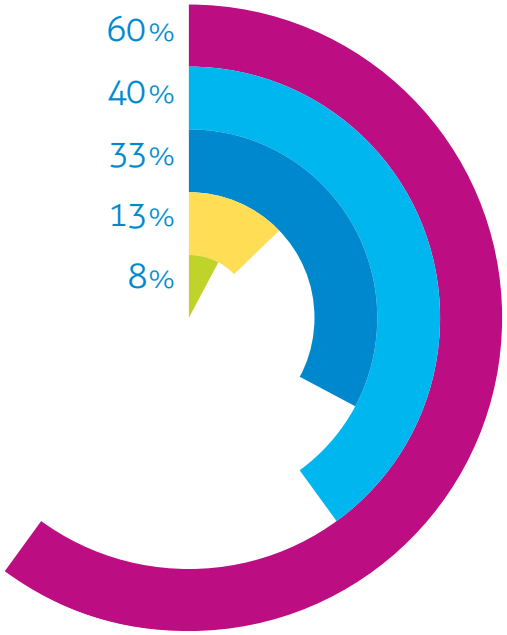
Pipeline Drug List

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
apremilast (Otezla®) (once-daily)	Celgene	PSO	Oral	Submitted - sNDA	Q4, 2018
rizatriptan	Intelgenx	Migraine treatment	Oral transmucosal	Submitted - 505(b)(2) NDA	Q4, 2018
tadalafil	Intelgenx	Erectile dysfunction	Oral	Submitted - 505(b)(2) NDA	Q4, 2018
canagliflozin (Invokana®)	Janssen	CVD risk reduction with T2DM	Oral	Submitted - sNDA	October 2018
canagliflozin/metformin (Invokamet®/Invokamet XR)	Janssen	CVD risk reduction with T2DM	Oral	Submitted - sNDA	October 2018
clobazam oral film	Aquestive	Lennox-Gastaut syndrome	Oral transmucosal	Submitted - 505(b)(2) NDA	October 2018
eltrombopag (Promacta®)	Novartis	Aplastic anemia (1st-line, with standard therapy)	Oral	Submitted - sNDA; Breakthrough Therapy; Orphan Drug; Priority Review	October 2018
trastuzumab (biosimilar to Genentech's Herceptin)	Merck/Samsung Bioepis	Breast cancer; Gastric/ gastroesophageal cancer	IV	Submitted - BLA	October 2018
pembrolizumab (Keytruda)	Merck	NSCLC (squamous, 1st-line, adjuvant, regardless of PD-L1 status)	IV	Submitted - sBLA; Priority Review	10/30/2018
epinephrine aerosol	Amphastar	Asthma	Inhaled	Submitted - 505(b)(2) NDA	November 2018
halobetasol propionate	Bausch Health	PSO	Topical	Submitted - 505(b)(2) NDA	November 2018
pasireotide (Signifor LAR®)	Novartis	Cushing's syndrome	IM	Submitted - sNDA	November 2018
oliceridine	Trevena	Acute pain	IV	Submitted - NDA; Breakthrough Therapy; Fast Track	11/02/2018
pegfilgrastim (biosimilar to Amgen's Neulasta)	Coherus	Neutropenia/leukopenia	SC	Submitted - BLA	11/02/2018
sufentanil	Acelrx	Acute pain	SL (medically supervised)	Submitted - 505(b)(2) NDA	11/02/2018
pembrolizumab (Keytruda)	Merck	HCC	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
oxycodone (abuse-deterrent)	Mallinckrodt	Pain	Oral	Submitted - 505(b)(2) NDA	11/16/2018
rifamycin	Cosmo	Gastroenteritis (traveler's diarrhea)	Oral	Submitted - NDA; Fast Track; Priority Review; Qualified Infectious Disease Product	11/16/2018
emapalumab	Swedish Orphan Biovitrum	Hemophagocytic lymphohistiocytosis	IV	Submitted - BLA; Breakthrough Therapy; Orphan Drug; Priority Review	11/20/2018

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
larotrectinib	Loxo Oncology	Solid tumors	Oral	Submitted - NDA; Breakthrough Therapy; Orphan Drug; Priority Review	11/26/2018
amifampridine	Catalyst	Lambert-Eaton myasthenic syndrome	Oral	Submitted - NDA; Breakthrough Therapy; Orphan Drug; Priority Review	11/28/2018
gilteritinib	Astellas	AML	Oral	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	11/29/2018
bupivacaine	Innocoll	Postsurgical pain	Implant	Submitted - NDA	11/30/2018
rituximab (biosimilar to Genentech's Rituxan)	Teva/Celltrion	RA; CLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis	IV	Submitted - BLA	11/30/2018
glasdegib	Pfizer	AML (untreated, with low-dose cytarabine)	Oral	Submitted - NDA; Orphan Drug; Priority Review	December 2018
itraconazole	Hedgepath	Fungal infections (systemic)	Oral	Submitted - 505(b)(2) NDA	Dec 2018-Jan 2019
atezolizumab (Tecentriq)	Genentech	NSCLC (nonsquamous, 1st-line, with bevacizumab & chemotherapy)	IV	Submitted - sBLA; Priority Review	12/05/2018
immunoglobulin (Bivigam®)	ADMA	Primary immunodeficiencies	IV	Submitted - sBLA	12/18/2018
trastuzumab (biosimilar to Genentech's Herceptin)	Teva/Celltrion	Breast cancer; Gastric/ gastroesophageal 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
venetoclax (Venclexta®)	Abbvie	AML	Oral	Submitted - sNDA; Breakthrough Therapy; Orphan Drug; Priority Review	12/25/2018
buprenorphine	Bluebird bio	Substance use disorder	SC	Submitted - 505(b)(2) NDA; Fast Track	12/26/2018
elotuzumab (Empliciti®)	Bristol-Myers Squibb	Multiple myeloma (≥ 2 prior therapies, with pomalidomide & dexamethasone)	IV	Submitted - sBLA; Orphan Drug; Priority Review	12/27/2018

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
glycerol phenylbutyrate (Ravicti®)	Horizon	Urea cycle disorders (ages < 2 months)	Oral	Submitted - sNDA; Fast Track	12/27/2018
astodrimer	Starpharma	Bacterial vaginosis (treatment & prevention)	Intravaginal	Submitted - NDA; Fast Track; Priority Review; Qualified Infectious Disease Product	12/28/2018
dasatinib (Sprycel®)	Bristol-Myers Squibb	ALL (Ph+, newly diagnosed, pediatrics, with chemotherapy)	Oral	Submitted - sBLA	12/28/2018
dexamethasone punctum plug	Ocular Therapeutix	Ocular pain/inflammation (post-operative)	Intraocular	Submitted - NDA	12/28/2018
pembrolizumab (Keytruda)	Merck	Merkel cell carcinoma (recurrent, locally advanced/metastatic)	IV	Submitted - sBLA; Breakthrough Therapy; Priority Review	12/28/2018
levetiracetam	Sun	Partial seizures	Oral	Submitted - 505(b)(2) NDA	January 2019
ospemifene (Osphena®)	Duchesnay	Vaginal atrophy	Oral	Submitted - sNDA	January 2019
levodopa	Acorda	Parkinson's disease	Inhaled	Submitted - 505(b)(2) NDA	01/05/2019
tacrolimus ER (Envarsus XR®)	Veloxis	Kidney transplant rejection (with immunosuppressants)	Oral	Submitted - sNDA	01/07/2019
pembrolizumab (Keytruda)	Merck	NSCLC (1st-line, monotherapy, EGFR & ALK negative)	IV	Submitted - sBLA; Priority Review	01/11/2019
romosozumab	Amgen	Osteoporosis treatment	SC	Submitted - BLA	01/11/2019
cabozantinib (Cabometyx®)	Exelixis	HCC	Oral	Submitted - sNDA; Orphan Drug	01/14/2019
sacituzumab govitecan	Immunomedics	Breast cancer	IV	Submitted - BLA; Breakthrough Therapy; Fast Track; Priority Review	01/18/2019
influenza virus vaccine (Fluzone® Quadrivalent)	Sanofi	Seasonal influenza vaccine (ages 6-35 months)	IM	Submitted - sBLA	01/28/2019
apomorphine	Sumitomo Dainippon	Parkinson's disease	Oral transmucosal	Submitted - 505(b)(2) NDA; Fast Track	01/29/2019
cladribine	Merck	MS (relapsing)	Oral	Submitted - NDA; Fast Track	01/30/2019
samidorphan/ buprenorphine	Alkermes	MDD	SL	Submitted - NDA; Fast Track; Priority Review	01/31/2019
insulin degludec/ liraglutide (Xultophy®)	Novo Nordisk	T2DM	SC	Submitted - sNDA	February 2019
ruxolitinib (Jakafi®)	Incyte	Graft versus host disease	Oral	Submitted - sNDA; Breakthrough Therapy; Orphan Drug; Priority Review	Feb-Mar 2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
aflibercept (Eylea®)	Regeneron	Diabetic retinopathy	Intraocular	Submitted - sNDA	Feb-Apr 2019
aflibercept prefilled-syrnge (Eylea)	Regeneron	Wet AMD	Intraocular	Submitted - sNDA	Feb-Apr 2019
ramucirumab (Cyramza®)	Eli Lilly	HCC	IV	Submitted - sBLA; Orphan Drug	Feb-May 2019
caplacizumab	Sanofi	Thrombotic thrombocytopenic purpura (TTP)	IV	Submitted - BLA; Fast Track; Orphan Drug; Priority Review	02/06/2019
iclaprim	Motif Bio	ABSSSI	IV	Submitted - NDA; Fast Track; Priority Review; Qualified Infectious Disease Product	02/14/2019
halobetasol propionate/ tazarotene	Bausch Health	PSO	Topical	Submitted - NDA	02/15/2019
pembrolizumab (Keytruda)	Merck	Melanoma (adjuvant, resected, high-risk stage 3)	IV	Submitted - sBLA; Breakthrough Therapy; Orphan Drug	02/15/2019
ravulizumab	Alexion	Paroxysmal nocturnal hemoglobinuria (PNH)	IV, SC	Submitted - BLA; Orphan Drug; Priority Review	02/18/2019
tagraxofusp	Stemline	Blastic plasmacytoid dendritic cell neoplasm	IV	Submitted - BLA; Breakthrough Therapy; Orphan Drug; Priority Review	02/21/2019
trifluridine/tipiracil (Lonsurf®)	Taiho Oncology	Gastric cancer (advanced/ metastatic, 2nd-line)	Oral	Submitted - sNDA; Priority Review	02/22/2019
afamelanotide	Clinuvel	Erythropoietic porphyria	Intradermal	Submitted - NDA; Fast Track; Orphan Drug	02/25/2019
loteprednol etabonate (sub-micron gel)	Bausch Health	Ocular pain/inflammation (post-operative)	Topical	Submitted - 505(b)(2) NDA	02/25/2019
turoctocog alfa pegol	Novo Nordisk	Hemophilia A	IV	Submitted - BLA	02/27/2019
macitentan (Opsumit®)	Actelion	Pulmonary hypertension (inoperable, chronic thromboembolic)	Oral	Submitted - sNDA	02/28/2019
midazolam spray	UCB	Seizure disorders (acute treatment)	Intranasal	Submitted - 505(b)(2) NDA; Fast Track; Orphan Drug	March 2019
siponimod	Novartis	MS (secondary progressive)	Oral	Submitted - NDA; Priority Review	March 2019
netarsudil/latanoprost	Aerie	Glaucoma/ocular hypertension	Topical	Submitted - 505(b)(2) NDA	03/14/2019
bremelanotide	AMAG	Female sexual arousal disorder	SC	Submitted - NDA	03/22/2019
olopatadine HCl/ mometasone furoate	Glenmark	Allergic rhinitis	Intranasal	Submitted - 505(b)(2) NDA	03/22/2019
sotagliflozin	Sanofi	T1DM	Oral	Submitted - NDA	03/22/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
meloxicam	Recro	Postsurgical pain	IV	Submitted - 505(b)(2) NDA	03/24/2019
diazepam	Neurelis	Repetitive seizures (aged ≥ 6 years)	Intranasal	Submitted - 505(b)(2) NDA; Fast Track; Orphan Drug	03/25/2019
aclidinium bromide (Tudorza® Pressair®)	AstraZeneca	COPD and CV safety	Inhaled	Submitted - sNDA	03/31/2019
aclidinium/formoterol	Circassia	COPD	Inhaled	Submitted - NDA	03/31/2019
glucagon	Eli Lilly	Hyperinsulinemia/ hypoglycemia	Intranasal	Submitted - NDA	Apr-Jul 2019
plasminogen (human)	Prometic Life Sciences	Hypoplasminogenemia	IV	Submitted - BLA; Fast Track; Orphan Drug; Priority Review	Apr-Sep 2019
metoclopramide	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
selinexor	Karyopharm	Multiple myeloma (penta-refractory)	Oral	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	04/05/2019
dolutegravir/lamivudine	GlaxoSmithKline	HIV-1 infection	Oral	Submitted - NDA; Priority review	04/18/2019
risankizumab	Abbvie	PSO	SC	Submitted - BLA	04/25/2019
alirocumab (Praluent®)	Regeneron	Major CV event reduction	SC	Submitted - sBLA	04/26/2019
adalimumab (biosimilar to Abbvie's Humira)	Merck/Samsung Bioepis	RA; AS; PSO; PsA; JIA; CD; UC	SC	Submitted - BLA	May 2019
dapagliflozin (Farxiga®)	AstraZeneca	T2DM with CKD	Oral	Submitted - sNDA	May 2019
exenatide (Bydureon®)	AstraZeneca	T2DM, CV outcomes	SC	Submitted - sNDA	May 2019
trastuzumab SC (Herceptin)	Genentech	Breast cancer	SC	Submitted - BLA	May 2019
cariprazine (Vraylar®)	Allergan	Bipolar depression	Oral	Submitted - sNDA	May-Jun 2019
tesamorelin (Egrifta®)	Theratechnologies	HIV lipodystrophy	SC	Submitted - sBLA	05/13/2019
nivolumab (Opdivo®)	Bristol-Myers Squibb	NSCLC (1st-line, with ipilmumab)	IV	Submitted - sBLA; Fast Track	05/20/2019
lumateperone (low-dose)	Intra-Cellular Therapies	Schizophrenia	Oral	Submitted - NDA; Fast Track	05/28/2019
NKTR-181	Nektar	Chronic low back pain	Oral	Submitted - NDA; Fast Track	05/28/2019
daratumumab (split-dose) (Darzalex®)	Janssen	Multiple myeloma	IV	Submitted - sBLA; Breakthrough Therapy; Orphan Drug	06/07/2019
AVXS-101	Novartis	Spinal muscular atrophy	IV	Submitted BLA; Breakthrough Therapy; Fast Track; Orphan Drug	06/18/2019
viaskin peanut	DBV	Peanut allergy	Transdermal	Submitted BLA; Breakthrough Therapy; Fast Track	06/21/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
celiprolol	Acer	Ehlers-Danlos syndrome	Oral	Sumbitted - NDA; Orphan Drug	06/28/2019
ibrutinib (Imbruvica®)	Abbvie	CLL (1st-line, with obinutuzumab)	Oral	Submitted - sNDA; Orphan Drug; Priority Review	July 2019
avatrombopag (Doptelet®)	Dova	Idiopathic thrombocytopenic purpura	Oral	Submitted - sNDA	07/04/2019
ferric maltol	Shield	Iron deficiency anemia	Oral	Submitted - NDA	08/01/2019
filgrastim (biosimilar to Amgen's Neupogen)	Tanvex	Neutropenia/leukopenia	IV, SC	Submitted - BLA	08/01/2019
esketamine	Janssen	MDD (resistent)	Intranasal	Submitted - NDA; Breakthrough Therapy; Fast Track	Q3, 2019
tenapanor	Ardelyx	IBS	Oral	Submitted - NDA	09/13/2019
erdafitinib	Janssen	Bladder cancer	Oral	Submitted - NDA; Breakthrough Therapy	09/18/2019
loteprednol etabonate	Kala	Dry eye	Topical	Submitted - 505(b)(2) NDA	Q4, 2019
filgrastim (biosimilar to Amgen's Neupogen)	Apotex	Neutropenia/leukopenia	IV, SC	Submitted - BLA	Pending
filgrastim (biosimilar to Amgen's Neupogen)	Adello	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 to Amgen's Neulasta)	Apotex	Neutropenia/leukopenia	SC	Submitted - BLA	Pending
abametapir	Dr. Reddy's	Head lice (aged ≥ 6 months)	Topical	Phase 3 - NDA	TBD
abatacept (Orencia®)	Bristol-Myers Squibb	Dermatomyositis; Lupus nephritis; Sjogren's syndrome	IV, SC	Phase 3 - sBLA; Orphan Drug	TBD
abicipar pegol	Allergan	Wet AMD	Intraocular	Phase 3 - BLA	TBD
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
albuterol (ProAir RespiClick®)	Teva	COPD	Inhaled	Phase 3 - sNDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
alicaforsen	Atlantic	UC	Rectal	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
alpelisib	Novartis	Breast cancer	Oral	Phase 3 - NDA	TBD
alpha galactosidase	Chiesi Farmaceutici	Fabry disease	IV	Phase 3 - BLA; Fast Track	TBD
alpha glucosidase (human, recombinant)	Sanofi	Pompe disease	IV	Phase 3 - BLA	TBD
amikacin liposomal (Arikayce®)	Insmed	CF	Inhaled	Phase 3 - sNDA; Orphan Drug	TBD
amilomotide	Novartis	Alzheimer's disease	IM, SC	Phase 3 - BLA	TBD
andolast	Mylan	Asthma	Inhaled	Phase 3 - NDA	TBD
anifrolumab	AstraZeneca	SLE	IV	Phase 3 - BLA; Fast Track	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 - sBLA	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)	Roche	Breast cancer; RCC	IV	Phase 3 - sBLA	TBD
avacopan	Chemocentryx	Antineutrophil cytoplasmic antibodies (ANCA) associated vasculitis	Oral	Phase 3 - NDA; Orphan Drug	TBD
avapritinib	Blueprint	GI stromal tumor	Oral	Phase 3 - NDA; Breakthrough Therapy; 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/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
bempedoic acid/ezetimibe	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
benralizumab (Fasenra®)	AstraZeneca	Nasal polyposis	SC	Phase 3 - sBLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Boehringer Ingelheim	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Kyowa Hakko Kirin	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Pfizer	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bimekizumab	UCB	PSO	IV	Phase 3 - BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease	Oral	Phase 3 - sNDA; Fast Track	TBD
brincidofovir	Chimerix	Adenovirus infection	Oral	Phase 3 - NDA; Fast Track	TBD
brolucizumab	Novartis	Wet AMD	Intraocular	Phase 3 - BLA	TBD
budesonide/formoterol	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
budesonide/formoterol fumarate/glycopyrronium	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
bupivacaine/meloxicam	Heron	Postsurgical pain	Instillation	Phase 3 - NDA; Breakthrough Therapy; Fast Track	TBD
C1-esterase inhibitor (Cinryze®)	Shire	Hereditary angioedema	SC	Phase 3 - sBLA	TBD
cabotegravir	GlaxoSmithKline	HIV-1 infection	IM, SC	Phase 3 - NDA	TBD
calcipotriene/ betamethasone dipropionate	MC2	PSO	Topical	Phase 3 - 505(b)(2) NDA	TBD
canagliflozin (Invokana)	Janssen	Diabetic nephropathy	Oral	Phase 3 - sNDA	TBD
cannabidiol (Epidiolex®)	GW	Tuberous sclerosis complex	Oral	Phase 3 - sNDA; Orphan Drug	TBD
capsaicin	Centrexion	Osteoarthritis	Intra-articular	Phase 3 - NDA; Fast Track	TBD
casimersen	Sarepta	Duchenne muscular dystrophy	IV	Phase 3 - NDA	TBD
cefiderocol	Shionogi	HAP	IV	Phase 3 - NDA	TBD
ceftolozane/tazobactam (Zerbaxa®)	Merck	HAP	IV	Phase 3 - sNDA; Fast Track; Qualified Infectious Diseases Product	TBD
cenicriviroc mesylate	Allergan	NASH	Oral	Phase 3 - NDA; Fast Track	TBD
cetuximab (Erbitux®)	Eli Lilly	Colorectal cancer (metastatic, BRAF-V600E+, with binimetinib & cetuximab)	IV	Phase 3 - sBLA; Breakthrough Therapy	TBD
citrulline	Asklepion	Acute lung injury	IV	Phase 3 - NDA; Orphan Drug	TBD
clascoterone	Cassiopea	Acne	Topical	Phase 3 - NDA	TBD
coversin	Akari	Paroxysmal nocturnal hemoglobinuria	SC	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
CTP-modified human growth hormone	Opko	Growth hormone deficiency	SC	Phase 3 - BLA; Orphan Drug	TBD
daprodustat	GlaxoSmithKline	Anemia due to CKD (dialysis dependent & independent)	Oral	Phase 3 - NDA	TBD
darvadstrocel	Tigenix	CD	IV	Phase 3 - BLA; Orphan Drug	TBD
dasiglucagon	Zealand	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 - NDA; Orphan Drug	TBD
dehydrated human amnion-chorion membrane	Mimedx	Achilles tendonitis; Plantar fasciitis	IV	Phase 3 - BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
depatuxizumab mafodotin	Abbvie	Brain cancer	IV	Phase 3 - BLA; Orphan Drug	TBD
deramanido	Otsuka	Tuberculosis	Oral	Phase 3 - NDA	TBD
derazantinib	Arqule	Biliary tract cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
diacerein	Castle Creek	Epidermolysis bullosa	Topical	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
dianhydrogalactitol	Delmar	Brain cancer	IV	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
difelikefalin	Cara	Pruritus	IV	Phase 3 - NDA; Breakthrough Therapy	TBD
dinutuximab beta	EUSA	Neuroendocrine tumors	SC	Phase 3 - BLA; Orphan Drug	TBD
diroximel fumarate	Biogen	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
donaperminogene seltoplasmid	Viromed	Diabetic foot ulcers	IM, Percutaneous	Phase 3 - BLA	TBD
dupilumab (Dupixent®)	Regeneron	Nasal polyposis	SC	Phase 3 - sBLA	TBD
durvalumab (Imfinzi®)	AstraZeneca	SCLC	IV	Phase 3 - sBLA	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 - NDA; Fast Track	TBD
dust mite immunotherapy	Stallergenes	Allergic rhinitis	SL	Phase 3 - BLA	TBD
ebola vaccine	Merck	Ebola infection prevention	IM	Phase 3 - BLA; Breakthrough Therapy	TBD
eculizumab (Soliris)	Alexion	Neuromyelitis optica (Devic's syndrome)	IV	Phase 3 - sBLA; Orphan Drug	TBD
efgartigimod	Argenx	Myasthenia gravis	IV, SC	Phase 3 - BLA; 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 (Orilissa®)	Abbvie	Uterine fibroids	Oral	Phase 3 - sNDA	TBD
empagliflozin (Jardiance®)	Boehringer Ingelheim	Diabetic nephropathy	Oral	Phase 3 - sNDA	TBD
epoetin alfa (biosimilar to Janssen's Procrit)	Novartis	Anemia due to CKD (dialysis dependent & independent)	IV, SC	Phase 3 - BLA	TBD
eptacog alfa (Novo Seven®)	Novo Nordisk	Factor VIII intolerance	IV	Phase 3 - BLA	TBD
eptinezumab	Alder	Migraine prevention	IV	Phase 3 - BLA	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 - NDA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Coherus	RA; JIA; AS; PSO; PsA	SC	Phase 3 - BLA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Samsung Bioepis	RA; JIA; AS; PSO; PsA	SC	Phase 3 - BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
etrolizumab	Roche	CD; UC	IV, SC	Phase 3 - BLA; Orphan Drug	TBD
fenfluramine	Zogenix	Dravet 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
ferric pyrophosphate (Triferic®)	Rockwell	Anemia due to CKD (dialysis-dependent)	IV	Phase 3 - sNDA	TBD
fevipiprant	Novartis	Asthma	Oral	Phase 3 - NDA	TBD
filgotinib	Gilead	RA	Oral	Phase 3 - NDA	TBD
fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®)	GlaxoSmithKline	Asthma	Inhaled	Phase 3 - sNDA	TBD
follitropin delta	Ferring	Female infertility	IV	Phase 3 - BLA	TBD
formoterol fumarate MDI	AstraZeneca	COPD	Inhaled	Phase 3 - 505(b)(2) NDA	TBD
fosfomycin	Nabriva	Complicated UTI	IV	Phase 3 - 505(b)(2) NDA	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
fusidic acid	Melinta	ABSSSI	Oral	Phase 3 - NDA	TBD
galcanezumab-gnlm (Emgality®)	Eli Lilly	Cluster headache (chronic)	SC	Phase 3 - sBLA; Fast Track	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 - NDA	TBD
givosiran	Alnylam	Porphyria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
glucagon pump	Xeris	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 - NDA; Orphan Drug	TBD
glycopyrrolate HFA MDI	AstraZeneca	Asthma; 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
guselkumab (Tremfya)	Janssen	PsA	SC	Phase 3 - sBLA	TBD
ibritumomab tiuxetan	Spectrum	DLBCL	IV	Phase 3 - BLA	TBD
icosapent ethyl (Vascepa®)	Amarin	Major CV event risk reduction	Oral	Phase 3 - sNDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
idasanutlin	Roche	AML	Oral	Phase 3 - NDA	TBD
idebenone	Santhera	Duchenne muscular dystrophy	Oral	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
immunoglobulin IV 10%	Octapharma	Thrombocytopenia	IV	Phase 3 - BLA	TBD
immunoglobulin IV 10%	Prometic Life	Primary immunodeficiencies	IV	Phase 3 - BLA	TBD
inclisiran	The Medicines Company	Dyslipidemia/ hypercholesterolemia	SC	Phase 3 - NDA; Orphan Drug	TBD
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
infliximab (biosimilar to Janssen's Remicade)	Nichi-Iko	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
ixekizumab (Taltz)	Eli Lilly	Axial spondyloarthritis	SC	Phase 3 - sBLA	TBD
lasmiditan	Eli Lilly	Migraine treatment	Oral	Phase 3 - NDA	TBD
lefamulin	Nabriva	CAP	IV, Oral	Phase 3 - NDA; Fast Track; Qualified Infectious Disease 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	TBD	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	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 - 505(b)(2) NDA; Orphan Drug	TBD
lisocabtagene maraleucel	Celgene	DLBCL	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug; Regenerative Medicine Advanced Therapy	TBD
lumasiran	Alnylam	Hyperoxaluria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
lumateperone	Ironwood	Gastroesophageal reflux disease	Oral	Phase 3 - 505(b)(2) NDA	TBD
luspatercept	Acceleron	Anemia; Myelodysplastic syndrome	SC	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
mannitol	Pharmaxis	CF	Inhaled	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
maribavir	Shire	Cytomegalovirus infection treatment	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
masitinib mesylate	AB Science	Alzheimer's disease; Asthma	Oral	Phase 3 - NDA	TBD
mepolizumab (Nucala®)	GlaxoSmithKline	Nasal polyposis	SC	Phase 3 - sBLA	TBD
meropenem/vaborbactam (Vabomere®)	Melinta	HAP; Septicemia	IV	Phase 3 - sNDA	TBD
metachromatic leukodystrophy gene therapy	Orchard	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
minocycline	Foamix	Acne; Rosacea	Topical	Phase 3 - 505(b)(2) NDA	TBD
mirikizumab	Eli Lilly	UC	SC	Phase 3 - BLA	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	Bellerophon	PAH	Inhaled	Phase 3 - NDA; Orphan Drug	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 - NDA	TBD
nolasiban	Obseva	Female infertility	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	Breast cancer (adjuvant treatment)	Oral	Phase 3 - sNDA	TBD
olipudase alfa	Sanofi	Niemann-Pick disease	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
omalizumab (Xolair®)	Genentech	Nasal polyposis	SC	Phase 3 - sBLA	TBD
ondansetron	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ozanimod	Celgene	MS; UC	Oral	Phase 3 - NDA	TBD
palovarotene	Clementia	Fibrodysplasia ossificans progressiva	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
pegilodecakin	ARMO	Pancreatic cancer	SC	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
pexidartinib	Daiichi Sankyo	Pigmented villonodular synovitis	Oral	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
pimodivir	Janssen	Influenza (excluding vaccines)	Oral	Phase 3 - NDA; Fast Track	TBD
pitolisant	Harmony	Narcolepsy	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
plasminogen (human)	Kedrion	Ligneous conjunctivitis	Ophthalmic	Phase 3 - BLA; Orphan Drug	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	TBD
quizartinib	Daiichi Sankyo	AML	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; 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	Endometriosis; Uterine fibroids	Oral	Phase 3 - NDA	TBD
remestemcel-L	Mesoblast	Graft versus host disease (GVHD)	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 Disease Product	TBD
rilpivirine (long-acting)	Janssen	HIV-1 infection	IM	Phase 3 - NDA	TBD
rimegepant sulfate	Biohaven	Migraine treatment	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
risankizumab	Abbvie	CD	SC	Phase 3 - BLA; Orphan Drug	TBD
risperidone	Braeburn	Schizophrenia	SC Implant	Phase 3 - 505(b)(2) NDA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Amgen	RA; CLL/ SLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis	IV	Phase 3 - BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Pfizer	RA; CLL/ SLL; 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
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)	Oral	Phase 3 - NDA	TBD
RSV F nanoparticle vaccine	Novavax	Respiratory syncytial virus prevention	IM	Phase 3 - BLA; Fast Track	TBD
RVT-802	Enzyvant	Primary immunodeficiencies	TBD	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug; Regenerative Medicine Advanced Therapy	TBD
sacubitril/ valsartan (Entresto®)	Novartis	Heart failure (preserved ejection fraction)	Oral	Phase 3 - sNDA	TBD
samidorphan/olanzapine	Alkermes	Schizophrenia	Oral	Phase 3 - NDA	TBD
satralizumab	Roche	Neuromyelitis optica (Devic's syndrome)	SC	Phase 3 - BLA; Orphan Drug	TBD
seladelpar	Cymabay	Primary biliary cholangitis/hepatic fibrosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
selinexor	Karyopharm	Sarcoma	Oral	Phase 3 - NDA; Orphan Drug	TBD
selonsertib	Gilead	NASH	Oral	Phase 3 - NDA	TBD
semaglutide (Ozempic®)	Novo Nordisk	T2DM	Oral	Phase 3 - NDA	TBD
semaglutide (Ozempic)	Novo Nordisk	Obesity	SC	Phase 3 - sNDA	TBD
serlopitant	Menlo	Pruritus	Oral	Phase 3 - NDA	TBD
setmelanotide	Rhythm	Obesity	SC	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
sodium thiosulfate	Fennec	Chemotherapy-induced hearing loss	IV	Phase 3 - 505(b)(2) NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
sotagliflozin	Sanofi	T2DM	Oral	Phase 3 - NDA	TBD
sparsentan	Retrophin	Focal segmental glomerulosclerosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
spartalizumab	Novartis	Melanoma	IV	Phase 3 - BLA	TBD
sulopenem etzadroxil	Iterum	Uncomplicated UTI	IV, Oral	Phase 3 - NDA	TBD
tafamidis meglumine	Pfizer	Transthyretin amyloid (ATTR) cardiomyopathy	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
talacotuzumab (Erleada)	Janssen	Prostate cancer (metastatic, castration resistant)	IV	Phase 3 - sBLA	TBD
tanezumab	Pfizer	Osteoarthritis	IV, SC	Phase 3 - BLA; Fast Track	TBD
tasimelteon (Hetlioz®)	Vanda	Insomnia (jet-lag related); Smith-Magenis syndrome	Oral	Phase 3 - sNDA	TBD
tecarfarin	Espero	Anticoagulation	Oral	Phase 3 - NDA	TBD
tenapanor	Ardelyx	Hyperphosphatemia	Oral	Phase 3 - NDA	TBD
teprotumumab	Horizon	Graves' ophthalmopathy/ orbitopathy	IV	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
teriparatide recombinant human (follow-on to Eli Lilly's Forteo®)	Pfenex	Osteoporosis	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 Farmaceutici	Asthma	Oral	Phase 3 - NDA	TBD
tocilizumab (Actemra®)	Roche	Scleroderma	SC	Phase 3 - sBLA; Breakthrough Therapy	TBD
tralokinumab	AstraZeneca	Atopic dermatitis	SC	Phase 3 - BLA	TBD
transcon PEG growth hormone	Ascendis	Growth hormone deficiency	SC	Phase 3 - BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Tanvex	Breast cancer; Gastric/ gastroesophageal cancer	IV	Phase 3 - BLA	TBD
treprostinil (patch pump)	Steadymed	PAH	SC	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
triamcinolone acetonide	Clearside	Uveitis	Intraocular	Phase 3 - 505(b)(2) NDA	TBD
trigriluzole	Portage	Alzheimer's disease; Obsessive compulsive disorder	Oral	Phase 3 - NDA	TBD
ublituximab	TG Therapeutics	CLL/SLL	IV	Phase 3 - BLA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ubrogepant	Allergan	Migraine treatment	Oral	Phase 3 - NDA	TBD
udenafil	Allergan	Erectile dysfunction	Oral	Phase 3 - NDA	TBD
upadacitinib	Abbvie	Atopic dermatitis; RA	Oral	Phase 3 - NDA; Breakthrough Therapy	TBD
ursodeoxycholic acid	Retrophin	Primary biliary cholangitis	Oral	Phase 3 - NDA	TBD
ustekinumab (Stelara)	Janssen	UC	IV, SC	Phase 3 - sBLA; Orphan Drug	TBD
vadadustat	Akebia	Anemia due to CKD (dialysis-independent)	Oral	Phase 3 - NDA	TBD
valoctocogene roxaparvovec	Biomarin	Hemophilia A	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
vibegron	Roivant	Overactive bladder	Oral	Phase 3 - NDA	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
vonapanitase	Proteon	End-stage renal disease	Topical	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/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA; Fast Track	TBD
VX-659/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA	TBD
zanubrutinib	Beigene	Waldenstrom macroglobulinemia	Oral	Phase 3 - NDA; Fast Track; Orphan Drug	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
amisulpride	Acacia	Post-operative nausea/ vomiting	IV	CRL	TBD
buprenorphine spray	Insys	Acute pain	Oral Transmucosal	CRL	TBD
C1 esterase inhibitor, human, recombinant (Ruconest®)	Pharming	Hereditary angioedema prophylaxis	IV	CRL	TBD
canakinumab (Ilaris®)	Novartis	Atherosclerosis	SC	CRL	TBD
dasotraline	Sumitomo Dainippon	ADHD	Oral	Withdrawn	N/A
ibuprofen	Cumberland	Mild to moderate pain	IV	CRL	TBD
IVIG-SN	Green Cross	Primary immunodeficiencies	IV	CRL	TBD
mepolizumab (Nucala)	GlaxoSmithKline	COPD	IV, SC	CRL	TBD
oxycodone ER	Pain Therapeutics	Chronic pain	Oral	CRL	TBD
pembrolizumab (Keytruda)	Merck	SCCHN (2nd-line, recurrent or metastatic)	IV	Withdrawn	N/A
promethazine IR/ hydrocodone/ acetaminophen	Charleston	Moderate to severe pain	Oral	CRL	TBD
RI-002	ADMA	Primary immunodeficiencies	IV	CRL	TBD
stannsoporfin	Mallinckrodt	Hyperbilirubinemia	IM	CRL	TBD
ulipristal	Allergan	Uterine fibroids	Oral	CRL	TBD
volanesorsen	Akcea	Dyslipidemia/ hypercholesterolemia	SC	CRL	TBD

GLOSSARY

ABSSSI Acute Bacterial 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

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

CI Confidence Interval

CKD Chronic Kidney Disease

CLL Chronic Lymphocytic Leukemia

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

DPP-4 Dipeptidyl Peptidase 4

DR Delayed-Release

EDSS Expanded Disability Status Scale

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

HAM-D Hamilton Depression Rating Scale

HbA1c Hemoglobin A1c

HCC Hepatocellular Carcinoma

HCP Healthcare Professional

HCV Hepatitis C Virus

HER Human Epidermal Growth Factor Receptor

HER2 Human Epidermal Growth Factor Receptor 2

HFA Hydrofluoroalkane

HIT Heparin Induced Thrombocytopenia

HIV-1 Human Immunodeficiency-1 Virus

HTN Hypertension

HR Hazard Ratio

IBS Irritable Bowel Syndrome

IM Intramuscular

IV Intravenous

JIA Juvenile Idiopathic Arthritis

LDL-C Low-Density Lipoprotein Cholesterol

MADRS Montgomery–Åsberg Depression Rating Scale

MDD Major Depressive Disorder

MDI Metered Dose Inhaler

MS Multiple Sclerosis

GLOSSARY continued

N/A Not Applicable

NASH Non-Alcoholic Steatohepatitis

NDA New Drug Application

NHL Non-Hodgkin Lymphoma

NSAID Non-Steroidal Anti-Inflammatory Drug

NSCLC Non-Small Cell Lung Cancer

OS Overall Survival

PAH Pulmonary Arterial Hypertension

PARP poly(ADP-ribose) polymerase

PASI 50 Psoriasis Area and Severity Index ≥ 50%

PASI 70 Psoriasis Area and Severity Index ≥ 70%

PASI 90 Psoriasis Area and Severity Index ≥ 90%

PCI Percutaneous Coronary Intervention

PD-1 Programmed Death Protein 1

PD-L1 Programmed Death-Ligand 1

PDUFA Prescription Drug User Fee Application

PFS Progression-Free Survival

PGA Physicians Global Assessment

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

SCT Stem Cell Transplant

SGLT Sodium-Glucose coTransporter

SLE Systemic Lupus Erythematosus

SLL Small Lymphocytic Lymphoma

sNDA supplemental New Drug Application

SOC Standard of Care

sPGA Static Physicians Global Assessment

SR Sustained-Release

SSRI Selective Serotonin Reuptake Inhibitors

SNRI Serotonin and Norepinephrine Reuptake Inhibitors

SSSI Skin and Skin Structure Infection

T1DM Type 1 Diabetes Mellitus

T2DM Type 2 Diabetes Mellitus

TBD To Be Determined

TNFα Tumor Necrosis Factor-alpha

UA Unstable Angina

UC Ulcerative Colitis

US United States

UTI Urinary Tract Infection

WHO World Health Organization

XR Extended-Release