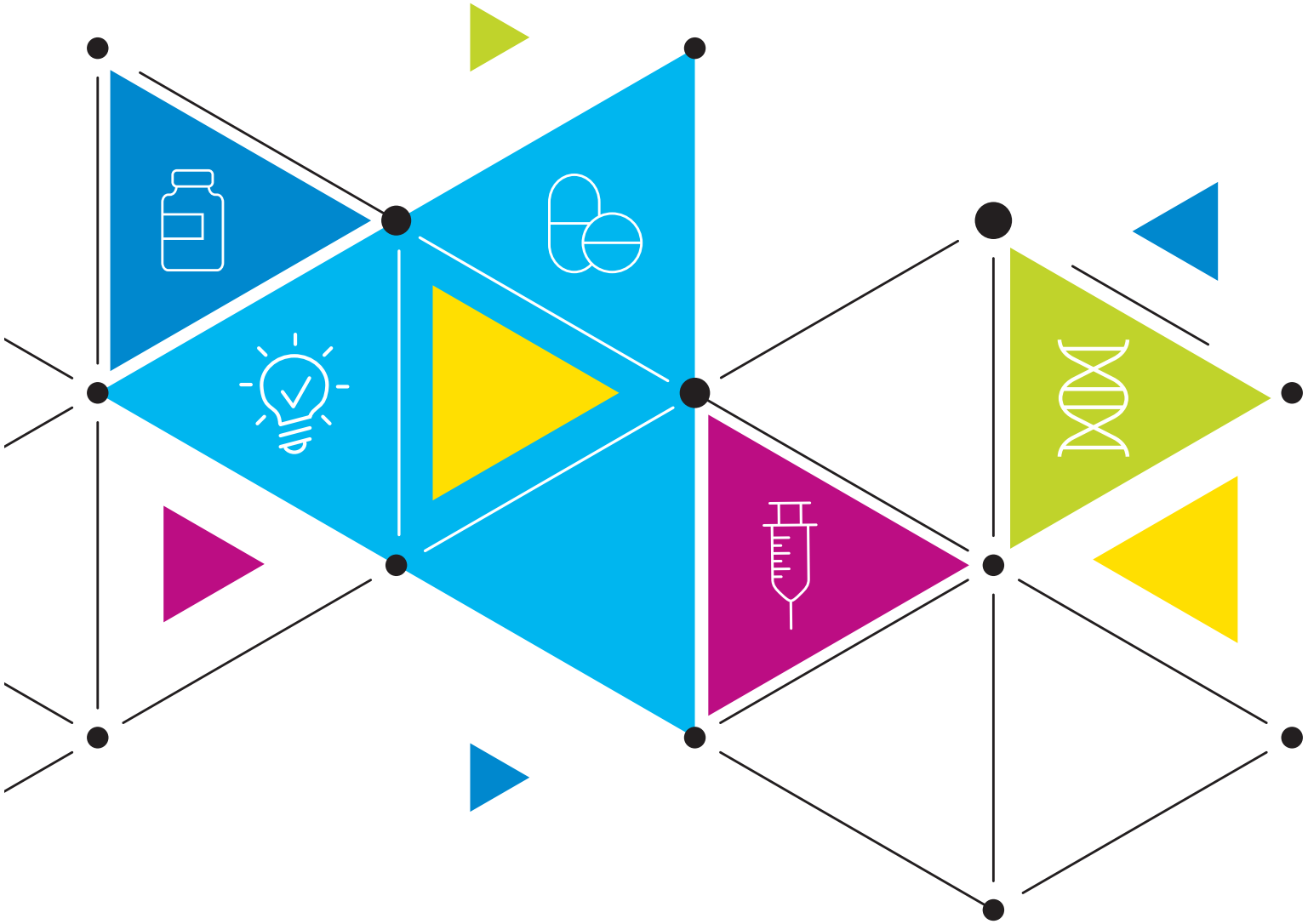
JULY 2019

MRx Pipeline

A view into upcoming specialty and traditional drugs



Magellan Rx MANAGEMENT℠

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INTRODUCTION

Welcome to the *MRx Pipeline*. In its third 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.

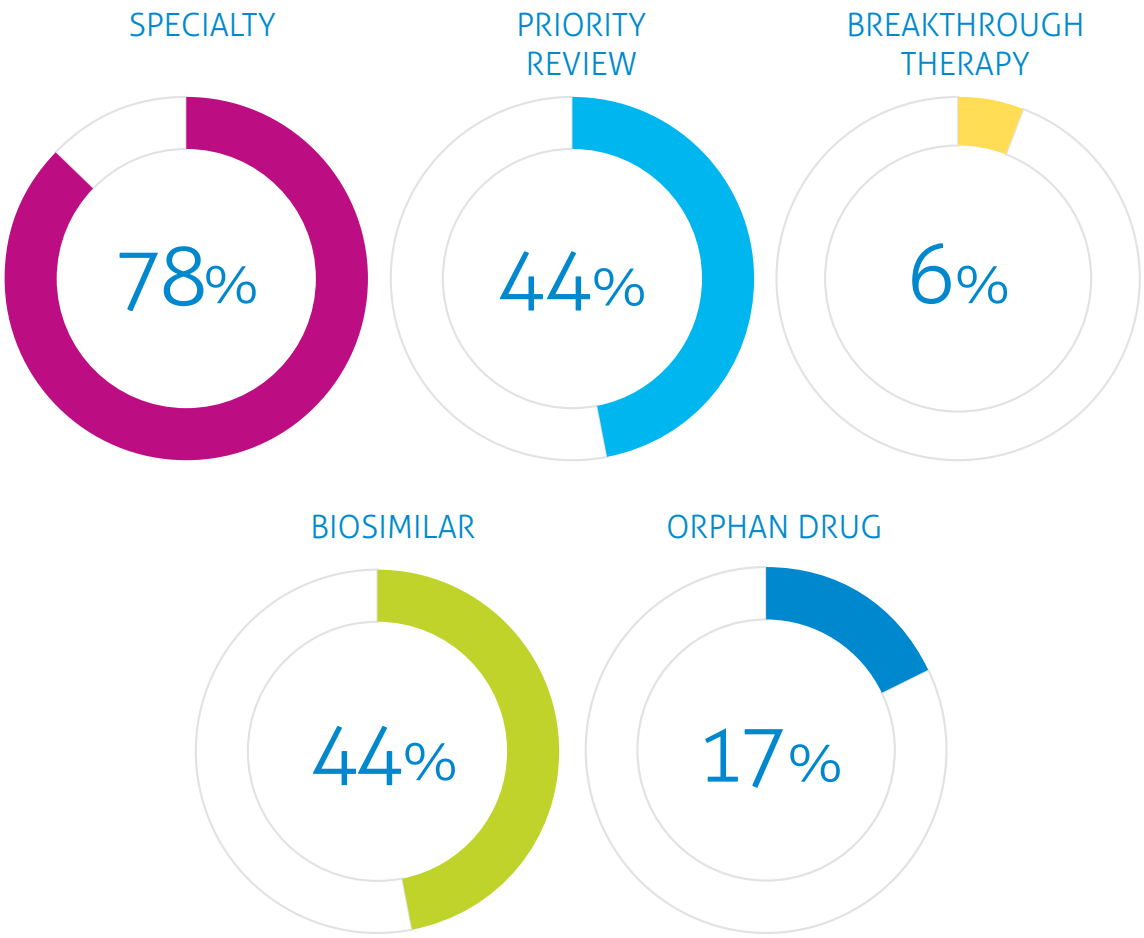
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year 2023. These figures are not specific to a particular commercial or government line of business; rather, they look at forecasted total US sales. Depending on a variety of factors, such as the therapeutic category, eventual approved FDA indications, population within the plan, and other indices, the financial impact could vary by different lines of business.

In the past few years, game changers, such as products in the hepatitis C field and chimeric antigen receptor (CAR)-T therapies, have revolutionized standard of care. As we look ahead, a continued trend toward the approval of specialty medications and the growth of biosimilars is expected, including first-time approvals for select biosimilars and market launches, digital therapeutics, and new treatment modalities using gene therapy. Noteworthy pipeline trends to watch in the upcoming quarters include the development of complex therapies, therapeutic options for rare hereditary diseases, oncology, immunology, neurology, and investigational agents for peanut allergies, and cystic fibrosis. Moreover, sprouting products for hemophilia, ophthalmology, hematology, and oral options for diabetes await on 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.

Ophthalmology

brolucizumab *intraocular*

Novartis

PROPOSED INDICATIONS

Wet age-related macular degeneration (AMD)

CLINICAL OVERVIEW

Brolucizumab is a vascular endothelial growth factor (VEGF) inhibitor.

Two similar double-blind, phase 3 trials, HAWK (n=1,082 eyes) and HARRIER (n=743 eyes), compared brolucizumab and aflibercept in patients with previously untreated wet AMD. In HAWK, eyes were randomized 1:1:1 to brolucizumab 3 mg, brolucizumab 6 mg, or aflibercept 2 mg, and in HARRIER, eyes were randomized 1:1 to brolucizumab 6 mg or aflibercept 2 mg. Following 3 monthly loading doses, brolucizumab was injected intravitreally every 12 weeks or every 8 weeks; however, if disease activity was identified in the every-12-weeks group, the dose was adjusted to every 8 weeks. Aflibercept was administered every 8 weeks from study onset. In both trials, at week 48, all arms of brolucizumab demonstrated non-inferiority compared to aflibercept in mean best-corrected visual acuity (BCVA) change from baseline. In HAWK, eyes treated with brolucizumab 3 mg, brolucizumab 6 mg, and aflibercept gained +6.1, +6.6, and +6.8 letters, respectively. Similarly, in HARRIER, eyes treated with brolucizumab 6 mg and aflibercept gained +6.9 and +7.6 letters, respectively. Significantly greater reductions in central subfield thickness, a secondary endpoint, were reported with brolucizumab 6 mg compared to aflibercept. In addition, at 48 weeks, significantly fewer brolucizumab-treated patients demonstrated disease activity, as measured by intraretinal fluid and/or subretinal fluid (24% in both studies versus 37% and 39% with aflibercept in HAWK and HARRIER, respectively). Approximately half of patients treated with brolucizumab 6 mg maintained every-12-week dosing. Similar safety profiles were seen with both agents.

PLACE IN THERAPY

AMD is a leading cause of vision loss in people ≥ 50 years of age. It is characterized by macular atrophy and formation of fatty deposits (drusen) under the macula. About 10% to 20% of cases progress to wet AMD (also called neovascular or exudative AMD) in which new blood vessels form behind the retina. The vessels are fragile and leak fluid and blood leading to scarring.

Intravitreal administration of VEGF inhibitors is considered first-line treatment. These agents include aflibercept (Eylea®), pegaptanib (Macugen®), and ranibizumab (Lucentis®), as well as off-label use of less costly bevacizumab (Avastin®). Biosimilars to Avastin (Mvasi™, Zirabev™) have been approved in the US; Mvasi is available and Zirabev is estimated to launch in Q4 2019. Approved VEGF inhibitors are administered by an HCP every 6 weeks (pegaptanib) or every 4 weeks (remaining agents). Brolucizumab is a single-chain antibody fragment (scFV) that inhibits all isoforms of VEGF-A. Its smaller molecule size may allow for better ocular tissue penetration resulting in every 8 or 12 week dosing and lower injection burden. In addition, unscheduled injections occurred 2.5 times less often with brolucizumab than aflibercept.

Pipeline VEGF inhibitors for wet AMD include conbercept administered intravitreally every 8 or 12 weeks. Additionally, brolucizumab is in late-stage development for macular edema due to diabetes or retinal vein occlusion and could compete with aflibercept and ranibizumab in these settings.

FDA APPROVAL TIMELINE

November 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$47	$241	$417	$554	$618

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

cabotegravir + rilpivirine *long-acting injectable (LAI)*
cabotegravir *oral*

Viiv

PROPOSED INDICATIONS
HIV-1 infection

CLINICAL OVERVIEW
Cabotegravir is an integrase strand transfer inhibitor (INSTI) analog of dolutegravir (Tivicay®); rilpivirine is a non-nucleoside reverse transcriptase inhibitor (NNRTI).

Two phase 3, open-label trials reported that switching to the LAI combination of cabotegravir + rilpivirine was non-inferior to continued use of a 3-drug oral ART regimen in virally suppressed HIV-1-infected adults. In ATLAS (n=308/arm), the 3-drug oral regimen contained 2 nucleoside reverse transcriptase inhibitors (NRTIs) plus an integrase strand transfer inhibitor (INSTI), NNRTI, or protease inhibitor (PI). The comparison therapy in the FLAIR trial (n=283/arm) was the single-tablet regimen abacavir/dolutegravir/lamivudine (ABC/DTG/3TC; Triumeq®). In both trials, cabotegravir + rilpivirine was non-inferior to the 3-drug regimen as measured by the proportion of participants with plasma HIV-1 RNA ≥50 copies/mL (primary endpoint) at week 48 (ATLAS, 1.6% versus 1%; FLAIR, 2.1% versus 2.5%; respectively). Incidence of confirmed virologic failure and resistant mutations were infrequent and were similar between the treatment arms. Cabotegravir + rilpivirine LAI was generally well tolerated. The most commonly reported adverse effect was injection site pain/reactions; 4 patients in ATLAS withdrew from the study due to injection site pain.

In ATLAS and FLAIR, patients received oral cabotegravir 30 mg + rilpivirine 25 mg once daily for 4 weeks to assess tolerability, after which patients received single 3 mL IM loading doses of cabotegravir LA 600 mg (200 mg/mL) + rilpivirine LA 900 mg (300 mg/mL), followed by 2 mL IM injections every 4 (± 1) weeks of cabotegravir LA 400 mg and rilpivirine LA 600 mg. Cabotegravir LA and rilpivirine LA were given as separate injections. Every-8-week dosing is being assessed in an ongoing open-label trial (ATLAS-2M).

PLACE IN THERAPY
If approved, cabotegravir + rilpivirine LAI will be the first LAI in the HIV armamentarium. It may require administration by an HCP and a 4-week lead-in period with daily self-administered oral cabotegravir + oral rilpivirine (Edurant®). The LAI combination had similar efficacy and drug resistance rates compared to the SOC 3-drug oral regimens, including the single-tablet ABC/DTG/3TC (Triumeq). Notably, viral replication assays showed the INSTI bictegravir, a component of single-tablet 3-drug regimen Biktarvy® (bictegravir/emtricitabine/tenofovir alafenamide), more effectively inhibits integrase mutant replication and retains potency against INSTI-resistant viruses compared to cabotegravir; clinical relevance has not been studied.

A once-monthly LAI could improve treatment compliance; however, a missed dose of a long-acting product could lead to sub-therapeutic drug levels and an increased risk of drug resistance. The LAI may be appropriate in patients for whom a daily ART regimen is problematic (e.g., social stigma) or those with comorbid conditions with multiple medications to coordinate. Moreover, cabotegravir + rilpivirine LAI has stable bioavailability, unlike Viiv's oral once-daily 2-drug complete regimen dolutegravir/rilpivirine (Juluca®), for which absorption can be impacted by food intake and potential drug interactions. Cabotegravir (LAI only), administered IM every 2 months, is being studied for pre-exposure prevention (PrEP) of HIV-1 infection.

FDA APPROVAL TIMELINE
December 27, 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$35	$87	$156	$302

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

Hematology

crizanlizumab *IV*

Novartis

PROPOSED INDICATIONS

Prevention of sickle cell disease (SCD)-related vaso-occlusive crises (VOCs)

CLINICAL OVERVIEW

SCD is a hereditary condition characterized by sticky, sickle-shaped RBCs. Extremely painful episodes, called vaso-occlusion crises (VOCs), result when the abnormal RBCs and leukocytes adhere to the vascular endothelium, causing blockage of small blood vessels and organ damage. Adhesion molecules, such as P-selectin, expressed on endothelial cells, play an important role in VOC. Crizanlizumab is a humanized monoclonal antibody that prevents vascular occlusion by blocking P-selectin.

The pivotal double-blind, placebo-controlled, phase 2 SUSTAIN trial (n=198) reported a significant 45.3% reduction in median annual crisis (VOC) rate in patients with SCD when treated with high-dose crizanlizumab (5 mg/kg of body weight) for 52 weeks (intent-to-treat annual VOC rate, 1.63 versus 2.98 for placebo). More than twice as many patients on high-dose crizanlizumab experienced no VOC during the trial (24 versus 11 for placebo). Moreover, clinically meaningful reductions in annual VOC rates were demonstrated with high-dose crizanlizumab, regardless of concomitant hydroxyurea (HU) use (50% reduction with HU and 32.1% without HU) or SCD genotype (34.6% reduction with HbSS and 50.5% with other genotypes). Incidence of arthralgia, diarrhea, pruritus, vomiting, and chest pain occurred at least twice as often with crizanlizumab than placebo. No significant effects on markers for hemolysis were detected.

In the clinical trial, patients received 2 loading doses of crizanlizumab 2 weeks apart, followed by maintenance doses every 4 weeks through week 50 for a total of 14 doses. Doses were administered IV over 30 minutes. While low-dose crizanlizumab (2.5 mg/kg) was also studied, it did not demonstrate statistically significant difference in annual VOC rate from placebo.

PLACE IN THERAPY

It is estimated that about 100,000 people in the US are diagnosed with SCD, with the highest prevalence among African Americans. VOC can have sudden onset and last hours to days. Frequency varies widely depending on specific genotype; HbSS is the most common and is associated with severe cases. VOCs are the most frequent cause of emergency department (ED) visits and hospitalization in patients with SCD.

Treatment options for SCD-related VOCs are limited. Aggressive hydration plus analgesics are uses to manage VOC episodes. Regular blood transfusions may prevent stroke in children with SCD and may reduce ED visits in select patients, but are associated with complications such as iron overload, infection, and alloimmunization. Currently, the oral antimetabolite HU is indicated to reduce the need for blood transfusions in patients with recurrent VOC; however, it is associated with leukemogenic and carcinogenic toxicities. Oral L-glutamine (Endari®), also approved for SCD, targets nicotinamide adenine dinucleotide and demonstrates SCD crisis reduction in patients stabilized on HU. Allogeneic bone marrow transplantation is curative, but patient selection is key due to associated risks and paucity of matched donors. If FDA-approved, crizanlizumab will be the first monoclonal antibody targeting P-selectin to prevent VOC in patients with SCD. Pfizer's injectable rivipansel is a pan-selectin inhibitor (E, L, and P) in phase 3 trials for treatment of SCD-related VOC in the hospital setting.

FDA APPROVAL TIMELINE

January 2020

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$29	$48	$84	$129

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

HIV/AIDS

emtricitabine/tenofovir alafenamide (Descovy®) *oral*

Gilead

PROPOSED INDICATIONS

HIV-1 infection pre-exposure prophylaxis (PrEP) to reduce the risk of acquiring HIV infection

Current indications for emtricitabine/tenofovir alafenamide (TAF) include use in combination with other ARVs for the treatment of HIV infection in adults and select pediatric patients.

CLINICAL OVERVIEW

Descovy is the fixed-dose combination of 2 HIV NRTIs, emtricitabine and TAF.

In the double-blind, phase 3, DISCOVER study, once-daily emtricitabine 200 mg/TAF 25mg (Descovy) was found to be non-inferior to once daily emtricitabine 200 mg/tenofovir disoproxil fumarate (TDF) 300 mg (Truvada®) for HIV PrEP in men who have sex with men (MSM) and transgender women at risk for sexually-acquired HIV infection (n=5,387). Over the course of the 2-year study, the incidence of HIV infection was 0.16/100 person-years (PY) with Descovy and 0.34/100 PY with Truvada. Among the 22 HIV infections reported in the study (both groups combined), 5 were likely acquired prior to the study. Intracellular drug levels were low or undetectable in 15 patients, and 2 patients had anticipated or intermediate levels. Furthermore, a significant difference in mean change in bone mineral density (BMD) was reported with Descovy compared to Truvada (spine, +0.5% versus -1.1%; hip, +0.2% versus -1%; respectively for both). Improvement in mean creatinine clearance (CrCl) was also seen with Descovy compared to a decrease with Truvada (+1.8 mL/min versus -2.3 mL/min); the difference was significant.

PLACE IN THERAPY

In the US, approximately 1.1 million people are living with HIV-1 infection. PrEP is the use of ARV therapy to prevent HIV infection in individuals at substantial risk of acquiring the disease. Daily PrEP has been shown to reduce this risk by up to 92%. A recent survey reported that during 2014 to 2017, the use of PrEP by MSM in select urban areas increased by about 500%; however, only about one-third of at-risk men reported using PrEP. In 2019, the US Department of Health and Human Services (DHHS) proposed a 4-pronged initiative to reduce HIV infection by 90% over the next 10 years; PrEP is among the strategies offered. Individuals at substantial risk of acquiring HIV infection include MSM, heterosexually active men and women, transgender women, and injection drug users.

In the US, Descovy and Truvada are approved in combination with other ARV for the treatment of HIV infection in adults and select pediatric patients. Approved for PrEP in adults in 2012 and for select adolescents in 2018, Gilead's Truvada became the first agent to be indicated for HIV PrEP in at-risk individuals and is considered safe and effective in most people when used in combination with safer sex practices. In clinical trials, Descovy has proven to be as effective as Truvada for PrEP, and since Descovy contains TAF rather than TDF, it may have a better safety profile regarding bone and renal toxicities compared to Truvada. If approved, Descovy will provide a second option for preventing HIV infection and have the potential to be the PrEP drug of choice in patients with renal impairment. Investigational cabotegravir is also in phase 3 trials as a long-acting injectable option for PrEP.

FDA APPROVAL TIMELINE

October 4, 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$1,338	$1,739	$2,089	$2,374	$2,537

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

Neurology

istradefylline *oral*

Kyowa Hakko Kirin

PROPOSED INDICATIONS

Parkinson's disease (PD) "off" episodes

CLINICAL OVERVIEW

The actions of dopamine receptors in the brain are countered by adenosine A2a receptors. Therefore, blocking adenosine A2a receptors is believed to enhance the activity of dopaminergic treatment, such as levodopa. Istradefylline is a selective adenosine A2a receptor antagonist that could modulate the "off" time of levodopa therapy without increasing dyskinesia.

Three 12-week, randomized, placebo-controlled trials evaluated istradefylline in patients with advanced PD receiving levodopa, with or without other PD agents, and experiencing motor fluctuations (n=1,419; North American trials). The primary endpoint of reduction in awake time spent in "off" state was met in only 1 of the trials, while other studies showed a significant improvement or a trend toward improvement in Unified Parkinson's Disease Rating Scale (UPDRS) subscore III, which measures motor function, and/or a trend toward reduction in daily "off" time. Istradefylline was generally well tolerated.

Istradefylline was studied as oral doses of 10 mg (1 study), 20 mg (3 studies), and 40 mg (2 studies) daily.

PLACE IN THERAPY

It is estimated that up to 1 million people in the US are diagnosed with PD, and carbidopa/levodopa (CD/LD) is a mainstay for treatment; however, over time its use is limited by "off" periods when effect diminishes, resulting in motor fluctuations. "Off" episodes, which affect a patient's ability to perform activities of daily living, are experienced by 40% to 60% of patients with PD, often worsening with disease progression. Dose modification and medication supplementation (e.g., dopamine agonists, catechol-O-methyl transferase [COMT] inhibitors, or monoamine oxidase [MAO] B inhibitors) may reduce "off" episodes, but these strategies may not be as helpful in advanced PD. New formulations are available, such as inhaled levodopa (Inbrija™) and continuous CD/LD via an intestinal infusion pump (Duopa™). Apomorphine (Apokyn®) is also available as a SC auto-injector for rescue treatment of sudden and severe "off" episodes.

While approved in Japan, istradefylline has met challenges in garnering approval in the US due to mixed results in clinical trials. If approved, it will be a first-in-class adenosine A2a receptor antagonist for treating PD. In 2017, development of Acorda's adenosine A2a receptor antagonist tozadenant was halted based on reports of serious or fatal cases of agranulocytosis; there are no published reports of this adverse effect associated with istradefylline to date. Other products in the pipeline for PD include the third generation COMT inhibitor opicapone, which is anticipated to be FDA-approved in April 2020. In addition, Sunovion is working toward resubmission for apomorphine sublingual film to the FDA after receiving a Complete Response Letter (CRL) in January 2019 requiring additional information.

FDA APPROVAL TIMELINE

August 27, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$2	$6	$6	$10	$14

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

Neurology

lasmiditan *oral*

Eli Lilly

PROPOSED INDICATIONS

Acute treatment of migraine in adults

CLINICAL OVERVIEW

Lasmiditan is a selective serotonin-1F (5-HT1F) receptor agonist that lacks vasoconstrictive activity.

Pooled data from the phase 3, double-blind SAMURAI and SPARTAN trials demonstrated lasmiditan 100 mg and 200 mg doses administered orally as needed for acute migraine attack led to significantly higher rates of pain relief and freedom from the most bothersome symptom (MBS; nausea, photophobia, or phonophobia) compared to placebo as early as 30 minutes post-dose ($p<0.05$). Additionally, a post-hoc analysis reported that efficacy regarding freedom from pain or MBS was not impacted by prior response or lack of response to triptans. Interim data from the ongoing, open-label, long-term GLADIATOR trial demonstrated efficacy for ≥ 12 months when used as needed. The most common treatment-related adverse effects were dizziness and somnolence.

PLACE IN THERAPY

Over 37 million people in the US, majority of whom are women, suffer from migraine headaches. Episodes can be debilitating, with pain lasting hours to days. Triptans are the current standard of care to treat acute, moderate to severe migraine attacks.

Triptans are contraindicated in patients with certain CV conditions. Lasmiditan is a first-in-class neurally acting anti-migraine agent (NAAMA) designed to alleviate migraine symptoms without vasoconstriction that is seen with other classes of antimigraine agents. Lasmiditan targets 5-HT1F receptors in the trigeminal pathway and has proven efficacy in treating migraine episodes in adults. If approved, it will provide a new mechanism in the migraine armamentarium and could address unmet needs in patients with migraine who also have CV risk factors. Two other potential competitors in the pipeline are the oral calcitonin gene-related peptide (CGRP) receptor antagonists, ubrogepant and rimegepant. Both have been submitted to the FDA seeking approval for acute treatment of migraine; FDA decisions are anticipated in December 2019 and February 2020, respectively. In 2018, 3 injectable monoclonal antibodies targeting the CGRP receptor (erenumab [Aimovig®], fremanezumab [Ajovy®], and galcanezumab [Emgality®]) were approved for migraine prophylaxis but do not carry indications for acute treatment.

FDA APPROVAL TIMELINE

November 14, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$13	$81	$149	$208	$268

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

luspatercept *sc*

Acceleron

PROPOSED INDICATIONS

Treatment of adults with beta-thalassemia-associated anemia who require red blood cell (RBC) transfusions

Treatment of adults with very low to intermediate-risk myelodysplastic syndromes (MDS)-associated anemia who have ring sideroblasts (MDS-RS) and require RBC transfusions

CLINICAL OVERVIEW

Luspatercept is a fusion protein that blocks transforming growth factor beta (TGF-beta) superfamily inhibitors of erythropoiesis, thereby promoting late-stage erythropoiesis.

Treatment with luspatercept plus best supportive care (RBC transfusion, iron chelation) was evaluated in the phase 3, double-blind, placebo-controlled BELIEVE trial in adults (n=336) with beta-thalassemia who required regular RBC transfusions. More luspatercept-treated patients (21.4% versus 4.5%) met the primary endpoint of ≥ 33% reduction in RBC transfusion burden with a reduction of ≥ 2 RBC units from baseline during weeks 13 to 24. The mean change in transfusion burden during this timeframe also favored luspatercept (difference from placebo, -1.35 RBC units). Luspatercept was generally well tolerated. Thromboembolic events (all grades) were reported at a higher rate with luspatercept (3.6% versus 0.9% placebo); grade 3/4 events were 0.9% and 0%, respectively.

The phase 3, double-blind, placebo-controlled MEDALIST trial evaluated luspatercept in adults (n=229) with very low- to intermediate-risk MDS who were RBC transfusion-dependent. Significantly more patients treated with luspatercept were RBC transfusion-independent for ≥ 8 weeks during weeks 1 through 24 (primary endpoint; 37.9% versus 13.2% with placebo). Approximately 40% of luspatercept-treated patients achieved durable erythroid responses up to 12 months. Significantly more patients treated with luspatercept experienced fatigue, diarrhea, asthenia, nausea, dizziness, and back pain. The open-label COMMAND trial comparing luspatercept and epoetin alfa as first-line treatment for MDS is ongoing.

In both trials, the initial dose of luspatercept was 1 mg/kg SC every 21 days. Dosage could increase up to 1.25 mg/kg in BELIEVE and 1.75 mg/kg in MEDALIST.

PLACE IN THERAPY

Symptomatic cases of beta thalassemia are estimated to occur in 1 in 100,000 individuals and are most often found in Mediterranean, Middle Eastern, African, Asian, and Indian populations. If only 1 of 2 genes (thalassemia minor) is affected, the resulting mild to moderate anemia typically does not require specific treatment. When both genes are affected (thalassemia major), complications such as pulmonary hypertension, heart failure, aplastic crisis, osteoporosis, and poor dental health arise. Treatments for this more severe form include long-term transfusion therapy, iron chelation, splenectomy, HSCT, and supportive care.

In the US, an estimated 10,000 cases of MDS are diagnosed each year, most often in people in their 70s. MDS-RS accounts for 10% to 15% of MDS cases. It is characterized by low RBC counts and normal WBC and platelet levels, and rarely progresses to leukemia. HSCT is the only curative treatment for MDS and is typically the treatment of choice in younger, relatively healthy patients. Alternative therapies include supportive measures (e.g., epoetin alfa, RBC transfusion, iron chelation), chemotherapy, and immune therapy.

Luspatercept is a first-in-class erythroid maturation agent. Unlike erythropoietin, which acts during earlier stages of erythrocyte proliferation, luspatercept regulates late-stage RBC development. While luspatercept is not a curative therapy, it has the potential to decrease the need for blood transfusions, iron chelation, and hospitalization in patients with beta-thalassemia or MDS, in turn increasing QOL in patients who have inadequate responses to current therapy. Also in the hematology pipeline, Bluebird Bio's lentiviral beta-globin gene transfer therapy, which produces a fully functional human beta-globin gene, is expected to be submitted to the FDA for transfusion-dependent beta-thalassemia by the end of 2019. Luspatercept is also in phase 2 trials for non-transfusion-dependent beta-thalassemia and for myelofibrosis.

luspatercept *SC (continued)*

FDA APPROVAL TIMELINE

Beta-thalassemia: December 4, 2019

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

Myelodysplastic syndromes: April 3, 2020

✓ Fast Track ✓ Orphan Drug

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$83	$215	$340	$462

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

Immunology

nintedanib (Ofev®) *oral*

Boehringer Ingelheim

PROPOSED INDICATIONS

Systemic sclerosis (scleroderma)-associated interstitial lung disease (ILD)

Nintedanib is currently approved for the treatment of idiopathic pulmonary fibrosis.

CLINICAL OVERVIEW

Nintedanib limits tumor angiogenesis. It inhibits the following receptor tyrosine kinases (RTK): vascular endothelial growth factor (VEGF), platelet-derived growth factor (PDGF), fibroblast growth factor (FGF), and Fms-like tyrosine kinase-3 (FLT3), as well as select non-RTKs (Src, Lck, and Lyn).

The phase 3, double-blind, SENSCIS trial evaluated efficacy and safety of nintedanib compared to placebo in patients (n=576) with systemic sclerosis-associated ILD affecting ≥ 10% of the lungs. A significant difference in annual rate of decline in forced vital capacity (FVC) of 41 mL/year (p=0.04) was demonstrated between the 2 groups favoring nintedanib. However, at week 52, there were no significant differences between the 2 groups regarding change from baseline in skin fibrosis (modified Rodnan skin score, −0.21 [95% CI, −0.94 to 0.53; p=0.58]) and QOL (St. George's Respiratory Questionnaire [SGRQ] total score, 1.69 [95% CI, −0.73 to 4.12]). Treatment with nintedanib was associated with GI adverse effects.

Nintedanib was studied at a dosage of 150 mg orally twice daily.

PLACE IN THERAPY

Systemic sclerosis is an autoimmune disorder characterized by abnormal connective tissue growth. It can affect only the skin (localized) or involve blood vessels and internal organs (systemic). Incidence is approximately 20 cases per million people in the US. It occurs in about 4 times as many women as men. Onset is typically between the ages of 25 to 55 years. Severity ranges from mild to life-threatening, depending on the areas and extent of the body affected. Systemic disease is considered *limited* (progressing slowly and confined to hands and face) or *diffuse* (developing more rapidly with internal organ involvement). Prompt and proper diagnosis and treatment may minimize the symptoms and lessen the potential for irreversible damage.

Within 3 years of diagnosis, about 25% of patients with systemic sclerosis develop significant pulmonary involvement which plays a key role in mortality in this patient population. Currently there is no treatment for ILD associated with systemic sclerosis. Nintedanib has the potential to expand its pulmonary use into the ILD arena. Similarly, the oral immunomodulator pomalidomide (Pomalyst®) is in phase 2 trials for systemic sclerosis-related ILD.

FDA APPROVAL TIMELINE

September 18, 2019

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$1,085	$1,143	$1,210	$1,286	$1,372

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

semaglutide *oral*

Novo Nordisk

PROPOSED INDICATIONS

Type 2 diabetes (T2DM) in adults

CLINICAL OVERVIEW

Semaglutide is a once-daily oral GLP-1 receptor agonist.

Oral semaglutide was evaluated in the PIONEER clinical program that includes eight phase 3 trials conducted in the US with over 5,800 adults with inadequately controlled T2DM. In PIONEER 4, at 26 weeks, oral semaglutide 14 mg was considered non-inferior to the GLP-1 agonist SC liraglutide for HbA1c reduction (1.2% versus 1.1%, respectively). Similar tolerability and discontinuation rates due to adverse effects were also seen (mild to moderate nausea, 20% versus 18%, respectively; discontinuation, 11% versus 9%, respectively). In addition, statistically greater reductions in body weight were reported with oral semaglutide (4.7 kg versus 3.2 kg, respectively; $p<0.0001$). In PIONEER 2, superiority was demonstrated versus the SGLT2 inhibitor empagliflozin (HbA1c reduction, 1.3% versus 0.9%, respectively) at 26 weeks. Oral semaglutide 7 mg and 14 mg also demonstrated significant HbA1c reduction over the oral DPP-4 inhibitor sitagliptin 100 mg at 26 weeks (1.3% versus 0.9%, respectively; PIONEER 3), and more patients achieved HbA1c < 7% with flexible doses of oral semaglutide (3 mg, 7 mg, 14 mg) than with sitagliptin at 52 weeks (58% versus 25%, respectively; PIONEER 7 [open-label]). Significantly greater reductions in HbA1c and total daily insulin doses were achieved when oral semaglutide was added to insulin (PIONEER 8). When compared to diet and exercise only, oral semaglutide significantly reduced HbA1c and more patients achieved HbA1c < 7% (PIONEER 1). Moreover, compared to placebo, oral semaglutide demonstrated efficacy in patients with moderate renal impairment (difference in HbA1c reduction, -0.8 percentage points; PIONEER 5) and no increase in major CV events (PIONEER 6, mean follow-up of 16 months).

In a 26-week, phase 2 trial, mean HbA1c reductions were similar between once-daily oral semaglutide 40 mg and once-weekly SC semaglutide 1 mg (Ozempic®) (−1.9% each). Notably, in the phase 2 trial, the oral semaglutide 40 mg dose is much higher than doses used in the phase 3 PIONEER studies (14 mg maximum). While rates of nausea with higher doses of oral semaglutide were not significantly higher than for SC semaglutide, rates of vomiting with oral doses over 10 mg were significantly higher (16% to 22% for oral versus 9% for SC). Slower dose escalation improved tolerability. The overall incidence of hypoglycemia was low and comparable between oral and SC formulations.

PLACE IN THERAPY

T2DM accounts for at least 90% of all diabetic cases. If approved, oral semaglutide will be the first oral GLP-1 agonist available in the US for T2DM. Similar efficacy and tolerability were demonstrated to the injectable GLP-1 agonist liraglutide and better effectiveness was seen versus agents in other antidiabetic classes (e.g., empagliflozin, sitagliptin). Dose-dependent nausea lessened with continued use. Data for use in combination with diet and exercise (± metformin) support its place in early-line treatment. Competition with injectable GLP-1 agonists, DPP-4 inhibitors, and SGLT2 inhibitors for second- and third-lines of therapy is also feasible, particularly since oral semaglutide is not associated with risk of genital infections, amputations, and bone fractures. Oral semaglutide may be preferred for T2DM patients who are needle-phobic and/or who would benefit from weight loss. Moreover, oral semaglutide does not increase risk of CV events, and unlike SLGT2 inhibitors, it may be appropriate in patients with moderate renal impairment.

FDA APPROVAL TIMELINE

September 20, 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$18	$279	$717	$1,192	$1,693

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. Select FDA biosimilar guidances are noted here. In January 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 pharmacists' abilities to substitute an interchangeable biosimilar for the reference product. 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. In May 2019, the agency released its final guidance on interchangeability. Several states had already enacted biosimilar substitution legislation.

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. In December 2018, the FDA announced its plans to transition a small subset of biologics currently approved as drugs under the Federal Food, Drug, and Cosmetics Act to be licensed as biologics. Starting in March 2020, drugs such as insulin and growth hormone will be deemed biologics and transition from the drug pathway to the biologics pathway. This new categorization can promote competition and access.

PLACE IN THERAPY

The patents of several biologic drugs are set to expire in the next few years, opening 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 two 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 marketplace.

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. The BAP contains four 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 23 biosimilars have received FDA approval. Of these, only 9 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)
Hyrimoz™ (adalimumab-adaz)	Sandoz	October 2018	-	Humira (Abbvie)
Udenyca® (pegfilgrastim-cbqv)	Coherus	November 2018	✓	Neulasta (Amgen)
Truxima® (rituximab-abbs)	Celltrion/Teva	November 2018	-	Rituxan® (Genentech)
Herzuma® (trastuzumab-pkrb)	Celltrion/Teva	December 2018	-	Herceptin (Genentech)
Ontruzant® (trastuzumab-dttb)	Samsung Bioepis/ Merck	January 2019	-	Herceptin (Genentech)
Trazimera™ (trastuzumab-qyyp)	Pfizer	March 2019	-	Herceptin (Genentech)
Eticovo™ (etanercept-ykro)	Samsung Bioepis/ Merck	April 2019	-	Enbrel (Amgen)
Kanjinti™ (trastuzumab-anns)	Amgen	June 2019	✓	Herceptin (Genentech)
Zirabev™ (bevacizumab-bvzr)	Pfizer	June 2019	-	Avastin (Genentech)
Ruxience™ (rituximab-pvvr)	Pfizer	July 2019	-	Rituxan (Genentech)
Hadlima™ (adalimumab-bwwd)	Samsung Bioepis/ Merck	July 2019	-	Humira (Abbvie)

* 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. In the US, it is estimated that 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 a $4.5 billion savings. A 2017 analysis by the Moran Company projects biosimilars could save the government an estimated $11.4 billion by 2027, but it would require the Centers for Medicare and Medicaid Services (CMS) to revise its reimbursement policy for biosimilars. Subsequently, in November 2017, the CMS revised its reimbursement policy. The CMS now issues a unique Healthcare Common Procedure Coding System (HCPCS) code to each individual biosimilar. Under this rule, Medicare Part B separately codes and pays 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.

Immunology

adalimumab (PF-06410293) *SC*

Pfizer

PF-06410293 is a biosimilar to Abbvie's Humira, a tumor necrosis factor alpha (TNF-α) 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

October-December 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$14,650	$15,557	$16,217	$16,693	$13,507

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

Blood Modifier
filgrastim *IV, SC*

Apotex, Kashiv, 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

Apotex (Grastofil)
Pending

Kashiv
Pending

Tanvex (TX01)
August 1, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$156	$129	$114	$102	$93

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

Immunology
infliximab (ABP-710) *IV*

Amgen

ABP-710 is a biosimilar to Janssen's tumor necrosis factor-alpha (TNF-α) inhibitor Remicade, indicated to treat rheumatoid arthritis (RA), ankylosing spondylitis (AS), plaque psoriasis (PSO), psoriatic arthritis (PsA), Crohn's disease (CD), and ulcerative colitis (UC).

FDA APPROVAL TIMELINE

October 17, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$ 2,788	$2,229	$1,864	$1,554	$1,228

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

Blood Modifier
pegfilgrastim *SC*

Lapelga and Ziextenzo are biosimilars to Amgen's Neulasta, a pegylated 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

Apotex (Lapelga)
Pending

Novartis (Ziextenzo)
October 3, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$3,186	$2,651	$2,218	$1,863	$1,575

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

Endocrine
teriparatide (PF708) *SC*

Pfenex

PF708 is an investigational follow-on to Eli Lilly's Forteo®, a recombinant human parathyroid hormone analog indicated for the treatment of postmenopausal, hypogonadal, and glucocorticoid-associated osteoporosis.

FDA APPROVAL TIMELINE

October 7, 2019

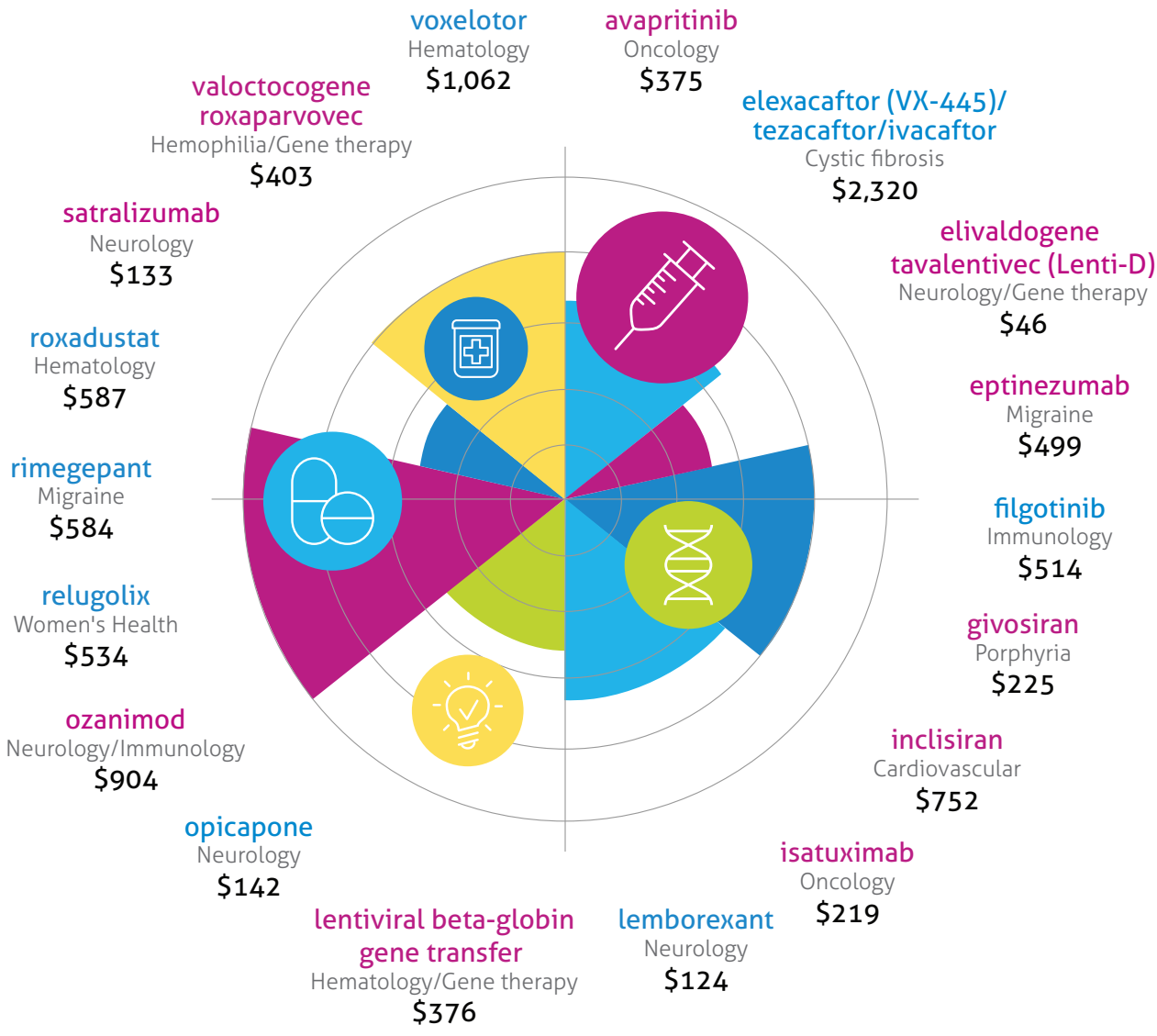
FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$615	$459	$361	$291	$239

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 2023, are displayed. The financials are projected total annual US sales, reported in *millions.*

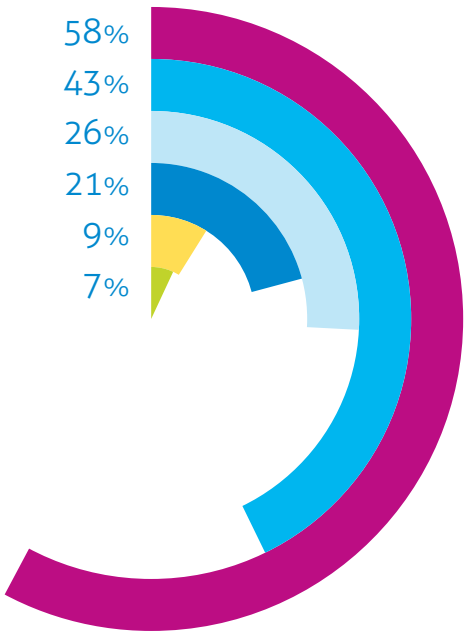


★ Specialty drug names appear in magenta throughout the publication.

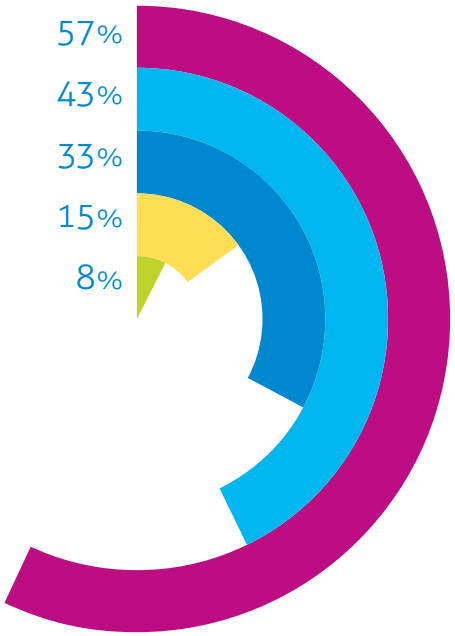
Pipeline Drug List

The pipeline drug list is an aerial outline of drugs with anticipated FDA approval through 2020. 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
lenalidomide (Revlimid®)	Celgene	Multiple myeloma (1st-line, in combination with bortezomib and dexamethasone)	Oral	Submitted – sNDA; Fast Track; Orphan Drug	July 2019
diazepam	Neurelis	Repetitive seizures (cluster/acute; ages ≥ 6 years)	Intranasal	Submitted – 505(b)(2) NDA; Fast Track; Orphan Drug	Jul-Sep 2019
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis	SC	Submitted – sBLA	August 2019
pitolisant	Harmony	Narcolepsy	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	August 2019
plasminogen (human)	Prometic Life Sciences	Hypoplasminogenemia	IV	Submitted – BLA; Fast Track; Orphan Drug; Priority Review	Aug-Sep 2019
pretomanid	Mylan	Tuberculosis (treatment resistant/nonresponsive)	Oral	Submitted – NDA; Fast Track; Orphan Drug; Priority Review	Aug-Sep 2019
benralizumab (Fasenra®) auto-injector	AstraZeneca	Eosinophilic asthma (self-administration)	SC	Submitted – sBLA	Aug-Dec 2019
filgrastim (biosimilar to Amgen's Neupogen)	Tanvex	Neutropenia/leukopenia	SC	Submitted – BLA	08/01/2019
pexidartinib	Daiichi Sankyo	Pigmented villonodular synovitis (symptomatic tenosynovial giant cell tumor)	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug; Priority Review	08/03/2019
cefiderocol	Shionogi	UTI (complicated)	IV	Submitted – NDA; Priority Review	08/14/2019
loteprednol etabonate 0.25%	Kala	Dry eye disease	Ophthalmic	Submitted – 505(b)(2) NDA	08/15/2019
tasimelteon (Hetlioz®)	Vanda	Jet lag disorder	Oral	Submitted – sNDA	08/16/2019
entrectinib	Genentech	NSCLC (metastatic, ROS1-positive); Solid tumors (NTRK fusion-positive, locally advanced or metastatic)	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug; Priority Review	08/18/2019
golodirsen	Sarepta	Duchenne muscular dystrophy	IV	Submitted – NDA; Orphan Drug; Priority Review	08/19/2019
lefamulin	Nabriva	CABP	IV, Oral	Submitted – NDA; Fast Track; Priority Review; QIDP	08/19/2019
upadacitinib	Abbvie	RA	Oral	Submitted – NDA; Priority Review	08/20/2019
istradefylline	Kyowa Hakko Kirin	Parkinson's disease ("off" episodes)	Oral	Submitted – NDA	08/27/2019
oxycodone ER (abuse- and alcohol-resistant)	Intellipharmaceutics	Chronic pain	Oral	Submitted – 505(b)(2) NDA; Fast Track	08/28/2019
NKTR-181	Nektar	Chronic low back pain	Oral	Submitted – NDA; Fast Track	08/29/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
smallpox vaccine	Bavarian Nordic	Smallpox	SC	Submitted – BLA; Priority Review	September 2019
rituximab (Rituxan)	Genentech	Granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA) (in combination with glucocorticoids, ages ≥ 2 years)	IV	Submitted – sBLA; Priority Review	Sep-Oct 2019
atezolizumab (Tecentriq®)	Genentech	NSCLC (1st-line, metastatic non-squamous, EGFR-negative, ALK-negative, in combination with abraxane and carboplatin)	IV	Submitted – sBLA	09/02/2019
fedratinib	Celgene	Myelofibrosis	Oral	Submitted – NDA; Orphan Drug; Priority Review	09/03/2019
glucagon pump	Xeris	Hyperinsulinemia/ hypoglycemia	SC	Submitted – 505(b)(2) NDA; Orphan Drug	09/10/2019
tenapanor	Ardelyx	IBS-C	Oral	Submitted – NDA	09/13/2019
nintedanib (Ofev)	Boehringer Ingelheim	Systemic sclerosis-associated interstitial lung disease	Oral	Submitted – sNDA; Fast Track; Orphan Drug; Priority Review	09/18/2019
mepolizumab (Nucala®) lyophilized powder	GlaxoSmithKline	Eosinophilic asthma (add-on therapy, ages 6-11 years)	SC	Submitted – sBLA	09/19/2019
canagliflozin (Invokana®)	Janssen	Diabetic nephropathy risk reduction with T2DM	Oral	Submitted – sNDA; Priority Review	09/20/2019
doravirine (Pifeltro®)	Merck	HIV-1 infection (switching from a stable ART regimen and virologically suppressed)	Oral	Submitted – sNDA	09/20/2019
doravirine/lamivudine/ tenofovir disoproxil fumarate (Delstrigo®)	Merck	HIV-1 infection (switching from a stable ART regimen and virologically suppressed)	Oral	Submitted – sNDA	09/20/2019
semaglutide	Novo Nordisk	T2DM	Oral	Submitted – NDA; Priority Review	09/20/2019
daratumumab (Darzalex®)	Janssen	Multiple myeloma (newly diagnosed, autologus SCT eligible, in combination with bortezomib, thalidomide, and dexamethasone [VTd])	IV	Submitted – sBLA; Priority Review	09/26/2019
icosapent ethyl (Vascepa®)	Amarin	Major CV event risk reduction	Oral	Submitted – sNDA; Priority Review	09/27/2019
lumateperone	Intra-Cellular Therapies	Schizophrenia	Oral	Submitted – NDA; Fast Track	09/27/2019
aflibercept (Eylea®) prefilled-syringe	Regeneron	Wet AMD	Intraocular	Submitted – sBLA	October 2019
treprostinil diolamine (Orenitram®)	United Therapeutics	Pulmonary hypertension (WHO Group 1)	Oral	Submitted – sNDA	October 2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
cetirizine	Pfizer	Urticaria	IV	Submitted – 505(b)(2) NDA	Oct-Nov 2019
lorcaserin (Belviq®)	Eisai	Obesity (long-term efficacy, removal of CV morbidity/mortality limitations)	Oral	Submitted – sNDA	Oct-Nov 2019
lorcaserin ER (Belviq XR®)	Eisai	Obesity (long-term efficacy, removal of CV morbidity/mortality limitations)	Oral	Submitted – sNDA	Oct-Nov 2019
onabotulinumtoxinA (Botox®)	Allergan	Lower limb spasticity (ages ≥ 2 years)	IM	Submitted – sBLA	Oct-Nov 2019
adalimumab (biosimilar to Abbvie's Humira)	Pfizer	RA; AS; PSO; PsA; JIA; CD; UC	SC	Submitted – BLA	Oct-Dec 2019
asenapine	Hisamitsu	Schizophrenia	Transdermal	Submitted – 505(b)(2) NDA	Oct-Dec 2019
influenza vaccine quadrivalent (Fluzone®) high-dose	Sanofi	Influenza prevention (ages ≥ 65 years)	IM	Submitted – sBLA	Oct-Dec 2019
pegfilgrastim (biosimilar to Amgen's Neulasta)	Novartis	Neutropenia/leukopenia	SC	Submitted – BLA	10/03/2019
emtricitabine/tenofovir alafenamide (Descovy)	Gilead	HIV-1 infection PrEP	Oral	Submitted – sNDA; Priority Review	10/04/2019
afamelanotide	Clinuvel	Erythropoietic porphyria	Intradermal	Submitted – NDA; Fast Track; Orphan Drug; Priority Review	10/06/2019
teriparatide recombinant, human (follow-on to Eli Lilly's Forteo)	Pfenex	Osteoporosis	SC	Submitted – 505(b)(2) NDA	10/07/2019
rivaroxaban (Xarelto®)	Janssen	VTE prevention in medically ill patients	Oral	Submitted – sNDA	10/14/2019
diroximel fumarate	Biogen	MS (relapsing)	Oral	Submitted – 505(b)(2) NDA	10/17/2019
infliximab (biosimilar to Janssen's Remicade)	Amgen	RA; AS; PSO; PsA; CD; UC	IV	Submitted – BLA	10/17/2019
triamcinolone ER (Zilretta®)	Flexion	Osteoarthritis of the knee (repeat dosing)	Intraarticular	Submitted – sNDA	10/17/2019
minocycline	Foamix	Acne (ages ≥ 9 years)	Topical	Submitted – 505(b)(2) NDA	10/18/2019
romiplostim (Nplate®)	Amgen	Immune thrombocytopenia purpura (ITP) (resistant)	SC	Submitted – sBLA	10/18/2019
ustekinumab (Stelara®)	Janssen	UC	IV, SC	Submitted – sBLA	10/18/2019
ravulizumab-cwvz (Ultomiris®)	Alexion	Hemolytic uremic syndrome	IV	Submitted – sBLA; Orphan Drug; Priority Review	10/19/2019
triamcinolone acetonide	Clearside	Macular edema associated with uveitis	Intraocular	Submitted – 505(b)(2) NDA	10/19/2019
delafloxacin (Baxdela®)	Melinta	CABP	IV, Oral	Submitted – sNDA; Priority Review; QIDP	10/24/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
niraparib (Zejula®)	GlaxoSmithKline	Ovarian cancer (advanced, after ≥ 3 prior therapies, BRCA mutation or homologous recombination deficiency)	Oral	Submitted – sNDA; Fast Track; Orphan Drug; Priority Review	10/24/2019
darolutamide	Bayer	Prostate cancer (non-metastatic, castration-resistant)	Oral	Submitted – NDA; Fast Track; Priority Review	10/25/2019
naloxone	Adamis	Substance use disorder	IM	Submitted – 505(b)(2) NDA	10/31/2019
brolucizumab	Novartis	Wet AMD	Intraocular	Submitted – BLA; Priority Review	November 2019
dapagliflozin (Farxiga®)	AstraZeneca	T2DM CV outcomes	Oral	Submitted – sNDA	Nov-Dec 2019
dulaglutide (Trulicity®)	Eli Lilly	T2DM CV outcomes	SC	Submitted – sNDA	Nov-Dec 2019
methotrexate	Cumberland	PSO	SC	Submitted – NDA	Nov-Dec 2019
RVT-802 (postnatal thymus tissue transplant)	Enzyvant	Pediatric congenital athymia	TBD	Submitted – BLA; Breakthrough Therapy; Orphan Drug; Priority Review; RMAT	Nov-Dec 2019
rifabutin/amoxicillin/ pantoprazole	Redhill	*H. pylori* infection	Oral	Submitted – 505(b)(2) NDA; Fast Track; Priority Review; QIDP	11/02/2019
baloxavir marboxil (Xofluza®)	Genentech	Influenza vaccine (ages ≥ 12 years)	Oral	Submitted – sNDA	11/04/2019
testosterone undecanoate	Lipocine	Hypogonadism	Oral	Submitted – 505(b)(2) NDA	11/09/2019
lasmiditan	Eli Lilly	Migraine treatment	Oral	Submitted – NDA	11/14/2019
ethinyl estradiol/ levonorgestrel	Agile	Contraception	Transdermal	Submitted – 505(b)(2) NDA	11/15/2019
cenobamate	SK Biopharmaceuticals	Parital-onset seizure	Oral	Submitted – NDA	11/21/2019
riluzole film	Aquestive	Amyotrophic lateral sclerosis	Oral Transmucosal	Submitted – 505(b)(2) NDA; Orphan Drug	11/29/2019
ubrogepant	Allergan	Migraine treatment	Oral	Submitted – NDA	December 2019
vedolizumab (Entyvio®)	Takeda	UC	SC	Submitted – sBLA	Dec 2019 - Jan 2020
luspatercept	Acceleron	Beta-thalassemia	SC	Submitted – BLA; Fast Track; Orphan Drug; Priority Review	12/04/2019
tazarotene	Bausch Health	Acne	Topical	Submitted – 505(b)(2) NDA	12/20/2019
vernakalant	Correvio	Atrial fibrillation	IV	Submitted – NDA	12/24/2019
bupivacaine ER	Durect	Postsurgical pain	SC	Submitted – NDA	12/27/2019
cabotegravir	Viiv	HIV-1 infection	Oral	Submitted – NDA; Priority Review	12/27/2019
cabotegravir + rilpivirine (long-acting)	Viiv	HIV-1 infection	IM	Submitted – NDA; Priority Review	12/27/2019
lemborexant	Eisai	Insomnia	Oral	Submitted – NDA	12/27/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
AR101	Aimmune	Peanut allergy	Oral	Submitted – BLA; Breakthrough Therapy; Fast Track	January 2020
crizanlizumab	Novartis	Sickle cell disease-related vaso-occulsive crisis	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug; Priority Review	January 2020
budesonide/formoterol fumarate/glycopyrronium	AstraZeneca	COPD	Inhaled	Submitted – NDA	Jan-Feb 2020
osilodrostat	Novartis	Cushing's syndrome	Oral	Submitted – NDA; Orphan Drug	Jan-Mar 2020
cocaine 4% and 10%	Lannett	Anesthesia	Topical	Submitted – 505(b)(2) NDA	Jan-Jun 2020
insulin aspart (Fiasp®)	Novo Nordisk	T1DM (pediatrics)	SC	Submitted – sNDA	01/01/2020
semaglutide	Novo Nordisk	T2DM-related CV risk reduction	Oral	Submitted – NDA	01/20/2020
semaglutide (Ozempic)	Novo Nordisk	T2DM-related CV risk reduction	SC	Submitted – sNDA	01/20/2020
tazemetostat	Epizyme	Epithelial sarcoma	Oral	Submitted – NDA; Orphan Drug; Priority Review	01/23/2020
risperidone ER (microsphere)	Luye	Bipolar disorder; Schizophrenia	IM	Submitted – 505(b)(2) NDA	01/28/2020
leuprolide mesylate depot (ready-to-use)	Foresee	Prostate cancer	SC	Submitted – 505(b)(2) NDA	01/29/2020
empagliflozin/linagliptin/ metformin ER	Boehringer Ingelheim	T2DM	Oral	Submitted – NDA	February 2020
rimegepant	Biohaven	Migraine treatment	Oral	Submitted – NDA; Priority Review	February 2020
paclitaxel injection concentrate for suspension	Sun	Breast cancer	IV	Submitted – 505(b)(2) NDA	Feb-Mar 2020
avapritinib	Blueprint	GI stromal tumor (PDGFRA exon 18 mutant)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug: RTOR	Feb-Jun 2020
pembrolizumab (Keytruda®) - 6 week dosing regimen	Merck	New dosing regimen of 400 mg every 6 week dosing for gastric cancer, HCC, Hodgkin's lymphoma, melanoma, Merkel cell carcinoma, and primary mediastinal large B cell lymphoma	IV	Submitted – sBLA	02/18/2020
bempedoic acid	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted – NDA	02/20/2020
bempedoic acid/ezetimibe	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted – NDA	02/20/2020
eptinezumab	Alder Bio	Migraine prevention	IV	Submitted – BLA	02/21/2020
apalutamide (Erleada®)	Janssen	Prostate cancer (1st-line, metastatic, castration-resistant)	IV	Submitted – sBLA; RTOR	02/29/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
lamotrigine oral liquid	Eton	Partial seizures; Primary generalized tonic-clonic seizures; Lennox-Gastaut syndrome	Oral	Submitted – 505(b)(2) NDA	March 2020
naloxone	INSYS	Substance use disorder	Intranasal	Submitted – 505(b)(2) NDA	March 2020
bimatoprost SR	Allergan	Glaucoma/ocular hypertension	Intraocular	Submitted – NDA	Mar-Apr 2020
cysteamine bitartrate (Procysbi®) granules	Horizon	Nephropathic cystinosis	Oral	Submitted – NDA; Orphan Drug	Mar-Apr 2020
trifarotene	Galderma	Acne	Topical	Submitted – NDA	03/01/2020
elexacaftor (VX-445)/ tezacaftor/ivacaftor	Vertex	CF	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track	03/20/2020
ozanimod	Celgene	MS (relapsing)	Oral	Submitted – NDA	03/25/2020
ferric pyrophosphate (Triferic®)	Rockwell Medical	Anemia due to CKD (dialysis-dependent)	IV	Submitted – sNDA	03/27/2020
luspatercept	Acceleron	Myelodysplastic syndrome	SC	Submitted – BLA; Fast Track; Orphan Drug	04/03/2020
remimazolam	Cosmo	Anesthesia	IV	Submitted – NDA	04/03/2020
meningococcal conjugate vaccine	Sanofi	Meningococcal vaccines and other meningococcus-specific agents (antibacterial)	IM	Submitted – BLA	04/25/2020
opicapone	Neurocrine Biosciences	Parkinson's disease ("off" episodes)	Oral	Submitted – NDA	04/26/2020
isatuximab	Sanofi	Multiple myeloma	IV	Submitted – BLA; Orphan Drug	04/30/2020
neratinib maleate (Nerlynx®)	Puma Biotechnology	Breast cancer (HER2-positive metastatic, in combination with capecitabine, 3rd-line)	Oral	Submitted – sNDA	05/01/2020
dasotraline	Sumitomo Dainippon	Binge eating disorder	Oral	Submitted – NDA; Fast Track	05/14/2020
givosiran	Alnylam	Porphyria	SC	Submitted – NDA; Breakthrough Therapy; Orphan Drug	06/05/2020
teprotumumab	Horizon	Graves' ophthalmopathy/ orbitopathy	IV	Submitted – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	Jul-Sep 2020
enfortumab vedotin	Astellas	Bladder cancer	IV	Submitted – BLA; Breakthrough Therapy	07/16/2020
filgrastim (biosimilar to Amgen's Neupogen)	Apotex	Neutropenia/leukopenia	SC	Submitted – BLA	Pending
filgrastim (biosimilar to Amgen's Neupogen)	Kashiv	Neutropenia/leukopenia	IV, SC	Submitted – BLA	Pending
pegfilgrastim (biosimilar to Amgen's Neulasta)	Apotex	Neutropenia/leukopenia	SC	Submitted – BLA	Pending

PIPELINE DRUG LIST continued

abametapir	Dr. Reddy's	Head lice (ages ≥ 6 months)	Topical	Phase 3 – NDA	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	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 – BLA	TBD
albuterol (ProAir RespiClick®)	Teva	COPD	Inhaled	Phase 3 – sNDA	TBD
alicaforsen	Atlantic	Pouchitis	Rectal	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
andolast	Mylan	Asthma	Inhaled	Phase 3 – NDA	TBD
anifrolumab	AstraZeneca	SLE	IV	Phase 3 – BLA; Fast Track	TBD
apremilast (Otezla®) - once-daily	Celgene	PSO (scalp)	Oral	Phase 3 – sNDA	TBD
artesunate	La Jolla	Malaria	Not specified	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Orphan Drug	TBD
astodrimer	Starpharma	Bacterial vaginosis (treatment & prevention)	Intravaginal	Phase 3 – NDA; Fast Track; QIDP	TBD
ataluren	PTC	Duchenne muscular dystrophy	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
atezolizumab (Tecentriq)	Genentech	RCC	IV	Phase 3 – sBLA	TBD
avacopan	Chemocentryx	ANCA associated vasculitis	Oral	Phase 3 – NDA; Orphan Drug	TBD
avalglucosidase alfa	Sanofi	Pompe disease	IV	Phase 3 – BLA	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; Fast Track; Orphan Drug	TBD
baricitinib (Olumiant®)	Eli Lilly	Atopic dermatitis	Oral	Phase 3 – sNDA	TBD
BCX7353	Biocryst	Hereditary angioedema (HAE)	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
benralizumab (Fasenra)	AstraZeneca	Nasal polyposis	SC	Phase 3 – sBLA	TBD
benzoyl peroxide/tretinoin	Bausch Health	Acne	Topical	Phase 3 – 505(b)(2) NDA	TBD
benzoyl peroxide	Sol-gel	Rosacea	Topical	Phase 3 – 505(b)(2) NDA	TBD
benzoyl peroxide/tretinoin	Sol-gel	Acne	Topical	Phase 3 – 505(b)(2) NDA	TBD
bevacizumab	Outlook	Wet AMD	Intraocular	Phase 3 – BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
bevacizumab (biosimilar to Genentech's Avastin)	Bio-Thera Solutions	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 – BLA	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
biotin (high-dose)	Medday	MS	Oral	Phase 3 – NDA	TBD
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease; Biopolar disorder	Oral	Phase 3 – sNDA; Fast Track	TBD
budesonide (viscous suspension)	Takeda	Esophagitis	Oral	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Orphan Drug	TBD
budesonide HFA-MDI	AstraZeneca	COPD	Inhaled	Phase 3 – 505(b)(2) NDA	TBD
budesonide/formoterol MDI	AstraZeneca	COPD	Inhaled	Phase 3 – NDA	TBD
cabotegravir (long-acting)	Viiv	HIV-1 infection PrEP	IM	Phase 3 – NDA	TBD
calcipotriene/ betamethasone dipropionate	MC2	PSO	Topical	Phase 3 – 505(b)(2) NDA	TBD
cannabidiol (Epidiolex®)	GW	Tuberous sclerosis complex	Oral	Phase 3 – sNDA; Orphan Drug	TBD
cannabidiol oral solution	Insys	Dravet syndrome; Lennox-Gastaut syndrome; Infantile spasms	Oral	Phase 3 – NDA; Orphan Drug	TBD
cantharidin	Verrica	Molluscum contagiosum	Topical	Phase 3 – NDA	TBD
capmatinib	Novartis	NSCLC	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
capsaicin	Centrexion	Osteoarthritis (knee)	Intraarticular	Phase 3 – NDA; Fast Track	TBD
casimersen	Sarepta	Duchenne muscular dystrophy	IV	Phase 3 – NDA; Orphan Drug	TBD
cedazuridine/decitabine	Otsuka	Myelodysplastic syndrome	Oral	Phase 3 – NDA	TBD
cediranib	AstraZeneca	Ovarian cancer; Biliary tract cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
cenicriviroc mesylate	Allergan	NASH	Oral	Phase 3 – NDA; Fast Track	TBD
cetuximab (Erbitux®)	Eli Lilly	Colorectal cancer (metastatic, BRAFV600E+, with binimetinib & cetuximab)	IV	Phase 3 – sBLA; Breakthrough Therapy; Fast Track	TBD
citrulline	Asklepion	Acute lung injury	IV	Phase 3 – NDA; Orphan Drug	TBD
clascoterone	Cassiopea	Acne	Topical	Phase 3 – NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
CM-AT	Curemark	Autism spectrum disorders	Oral	Phase 3 – BLA; Fast Track	TBD
colasevalam	Ironwood	Gastroesophageal reflux disease	Oral	Phase 3 – 505(b)(2) NDA	TBD
conbercept	Chengdu Kanghong	Wet AMD	Intraocular	Phase 3 – BLA	TBD
cyclobenzaprine	Tonix	PTSD	SL	Phase 3 – 505(b)(2) NDA	TBD
dalcetrapib	Dalcor	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 – NDA	TBD
daprodustat	GlaxoSmithKline	Anemia due to CKD (dialysis-independent & dialysis-dependent)	Oral	Phase 3 - NDA	TBD
darvadstrocel	Takeda	CD	IV	Phase 3 – BLA; Orphan Drug	TBD
dasiglucagon	Zealand	T1DM-related severe hypoglycemia	SC	Phase 3 – NDA	TBD
dehydrated human amnion-chorion membrane	Mimedx	Achilles tendonitis; Plantar fasciitis; Osteoarthritis (knee)	IV	Phase 3 – BLA	TBD
deramanido	Otsuka	Tuberculosis	Oral	Phase 3 – NDA	TBD
dextromethorphan/ bupropion	Axsome	Alzheimer's disease; MDD	Oral	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Fast Track	TBD
dianhydrogalactitol	Delmar	Brain cancer	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
diazepam film	Aquestive	Seizure clusters	Oral transmucosal	Phase 3 – NDA; Orphan Drug	TBD
difelikefalin	Cara	Pruritus (hemodialysis-related)	IV	Phase 3 – NDA; Breakthrough Therapy	TBD
dihydroergotamine mesylate	Impel Neuropharma	Migraine treatment	Intranasal	Phase 3 – 505(b)(2) NDA	TBD
dinutuximab beta	EUSA	Neuroendocrine tumors	SC	Phase 3 – BLA; Orphan Drug	TBD
d-methylphenidate	Kempharm	ADHD	Oral	Phase 3 – NDA	TBD
donaperminogene seltoplasmid	Viromed	Diabetic peripheral neuropathy; Diabetic foot ulcers	IM	Phase 3 – BLA; RMAT	TBD
dupilumab (Dupixent®)	Regeneron	COPD; Esophagitis	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
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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
elivaldogene tavalentivec	Bluebird Bio	Cerebral adrenoleukodystrophy	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
empagliflozin (Jardiance®)	Boehringer Ingelheim	Diabetic nephropathy	Oral	Phase 3 – sNDA	TBD
EP-2101 vaccine	OSE Immunotherapeutics	NSCLC	SC	Phase 3 – NDA; Orphan Drug	TBD
epoetin alfa (biosimilar to Janssen's Procrit)	Novartis	Anemia due to CKD (dialysis-dependent)	IV, SC	Phase 3 – BLA	TBD
eptacog alfa (NovoSeven®)	Novo Nordisk	Factor VIII intolerance	IV	Phase 3 – sBLA	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 – NDA	TBD
estetrol/drospirenone	Mithra	Contraception	Oral	Phase 3 – NDA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Coherus	RA; PSO	SC	Phase 3 – BLA	TBD
etrasimod	Arena	UC	Oral	Phase 3 – NDA	TBD
fenfluramine (low-dose)	Zogenix	Dravet syndrome; Lennox Gastaut syndrome	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
fevipiprant	Novartis	Asthma	Oral	Phase 3 – NDA	TBD
fexapotide triflutate	Nymox	Benign prostatic hyperplasia	Intratumoral	Phase 3 – NDA	TBD
filgotinib	Gilead	RA; CD; UC	Oral	Phase 3 – NDA	TBD
fitusiran	Sanofi	Hemophilia A and B (with and without inhibitors)	SC	Phase 3 – NDA; Orphan Drug	TBD
fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®)	GlaxoSmithKline	Asthma	Inhaled	Phase 3 – sNDA	TBD
follitropin alfa (follow-on to EMD Serono's Gonal-F®)	Allergan	Reproductive disorder	SC	Phase 3 – 505(b)(2) NDA	TBD
follitropin alfa (follow-on to EMD Serono's Gonal-F)	Finox	Reproductive disorder	SC	Phase 3 – 505(b)(2) NDA	TBD
follitropin delta	Ferring	Female infertility	IV	Phase 3 – BLA	TBD
formoterol fumarate MDI	AstraZeneca	COPD	Inhaled	Phase 3 – 505(b)(2) NDA	TBD
fosmetpantotenate	Retrophin	Pantothenate kinase-associated neurodegeneration	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
fostemsavir	Viiv	HIV-1 infection	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track	TBD
fusidic acid	Melinta	ABSSSI	Oral	Phase 3 – NDA; QIDP	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 – NDA	TBD
GLPG1690	Galapagos	Idiopathic pulmonary fibrosis	Oral	Phase 3 – NDA; Orphan Drug	TBD
glycopyrrolate HFA MDI	AstraZeneca	COPD	Inhaled	Phase 3 – NDA	TBD
glycopyrronium bromide (Seebri® Neohaler®)	Sumitomo Dainippon	Asthma	Inhaled	Phase 3 – sNDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
grazoprevir/elbasvir (Zepatier®)	Merck	Hepatitis C 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
hydrocortisone (granules in capsule)	Diurnal	Adrenal insufficiency (pediatric)	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
ibrexafungerp	Scynexis	Fungal infections (systemic and non-systemic)	IV, Oral	Phase 3 – NDA; Fast Track; Orphan Drug; QIDP	TBD
iclaprim	Motif Bio	ABSSSI	IV	Phase 3 – NDA; Fast Track; QIDP	TBD
idasanutlin	Roche	AML	Oral	Phase 3 – NDA	TBD
idebenone	Santhera	Duchenne muscular dystrophy	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
idecabtagene vicluecel	Celgene	Multiple myeloma	IV	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	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 maleate/ mometasone furoate	Novartis	Asthma	Inhaled	Phase 3 – NDA	TBD
indacaterol/ glycopyrronium bromide/ mometasone furoate	Novartis	Asthma	Inhaled	Phase 3 – NDA	TBD
inebilizumab	Viela Bio	Neuromyelitis optica (Devic's syndrome)	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	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
insulin lispro/treprostinil	Eli Lilly	T1DM; T2DM	SC	Phase 3 – NDA	TBD
interferon-beta 1a	Faron	Acute respiratory distress syndrome	IV	Phase 3 – BLA; Fast Track	TBD
iodine I-131 monoclonal antibody	Actinium	Myeloablation prior to allogeneic HSCT to treat AML	IV	Phase 3 – BLA; Orphan Drug	TBD
ipatasertib	Genentech	Prostate cancer	Oral	Phase 3 – NDA	TBD
lentiviral beta-globin gene transfer	Bluebird Bio	Beta-thalassemia	IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
leronlimab	Cytodyn	HIV-1 infection	SC	Phase 3 – BLA; Fast Track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
levodopa/carbidopa (patch pump)	Mitsubishi Tanabe	Parkinson's disease	SC	Phase 3 – 505(b)(2) NDA	TBD
levoketoconazole	Strongbridge	Cushing's syndrome	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
linzagolix	Obseva	Endometriosis	Oral	Phase 3 – NDA	TBD
lisocabtagene maraleucel	Celgene	DLBCL	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug; RMAT	TBD
L-lactic acid/citric acid/ potassium bitartrate	Evofem	Contraception; Chlamydia and gonorrhea prevention (women)	Intravaginal	Phase 3 – NDA; Fast Track; QIDP	TBD
lorecivivint	Samumed	Osteoarthritis (knee)	Intraarticular, Intradiscal	Phase 3 – NDA	TBD
lumasiran	Alnylam	Hyperoxaluria	SC	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
lumateperone	Intra-Cellular Therapies	Bipolar disorder	Oral	Phase 3 – NDA	TBD
lutetium 177Lu-PSMA-617	Endocyte	Prostate cancer	IV	Phase 3 – NDA	TBD
margetuximab	Macrogenics	Breast cancer	IV	Phase 3 – BLA; Fast Track	TBD
maribavir	Shire	Cytomegalovirus infection treatment	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
masitinib mesylate	AB Science	Asthma; Alzheimer's disease; Amyotrophic lateral sclerosis; Mastocytosis	Oral	Phase 3 – NDA	TBD
mavacamten	Myokardia	Obstructive hypertrophic cardiomyopathy	Oral	Phase 3 – NDA; Orphan Drug	TBD
melflufen	Oncopeptides	Multiple myeloma	IV	Phase 3 – NDA; Orphan Drug	TBD
meloxicam/rizatriptan	Axsome	Migraine treatment	Oral	Phase 3 – 505(b)(2) NDA	TBD
meropenem/vaborbactam (Vabomere®)	Melinta	HAP; Septicemia	IV	Phase 3 – sNDA; QIDP	TBD
metachromatic leukodystrophy gene therapy	Orchard	Metachromatic leukodystrophye	IV	Phase 3 - BLA; Orphan Drug	TBD
microbiota suspension	Rebiotix	Recurrent *Clostridium difficile* infection	Rectal	Phase 3 – sBLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
minocycline	Foamix	Rosacea	Topical	Phase 3 – 505(b)(2) NDA	TBD
minocycline	Citius	Catheter-related bloodstream infections	IV	Phase 3 – 505(b)(2) NDA; Fast Track; QIDP	TBD
mirikizumab	Eli Lilly	PSO; UC	SC	Phase 3 – BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
mitomycin	Urogen	Bladder cancer	Intravesical	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
molgramostim	Savara	Pulmonary alveolar proteinosis	Inhaled	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
molindone	Supernus	ADHD	Oral	Phase 3 – 505(b)(2) NDA; Fast Track	TBD
nifurtimox	Bayer	Chagas disease	Oral	Phase 3 – NDA; Orphan Drug	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 – NDA	TBD
nitric oxide	AIT	Bronchiectasis	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
octreotide	Chiasma	Acromegaly	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
olanzapine/samidorphan	Alkermes	Schizophrenia; Bipolar disorder (weight gain mitigation)	Oral	Phase 3 – NDA	TBD
olaparib (Lynparza®)	AstraZeneca	Breast cancer (adjuvant treatment)	Oral	Phase 3 – sNDA; Breakthrough Therapy	TBD
oliceridine	Trevena	Acute pain	IV	Phase 3 – NDA; Fast Track	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 ER (once-daily)	Redhill	Gastroenteritis	Oral	Phase 3 – 505(b)(2) NDA	TBD
oportuzumab monatox	Sesen Bio	Bladder cancer	Intravesical	Phase 3 – BLA; Fast Track	TBD
oxycodone ER	Egalet	Chronic low back pain; Moderate to severe pain	Oral	Phase 3 – 505(b)(2) NDA; Fast Track	TBD
oxymetazoline	Revitalid	Ptosis	Ophthalmic	Phase 3 – 505(b)(2) NDA	TBD
ozanimod	Celgene	CD; UC	Oral	Phase 3 – NDA	TBD
palovarotene	Clementia	Fibrodysplasia ossificans progressiva	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
pegunigalsidase alfa	Chiesi	Fabry disease	IV	Phase 3 – BLA; Fast Track	TBD
pemigatinib	Incyte	Biliary tract cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
PF-06651600	Pfizer	Alopecia areata	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
pimodivir	Janssen	Influenza treatment	Oral	Phase 3 – NDA; Fast Track	TBD
pineapple proteolytic enzymes extract	Mediwound	Burn injury	Topical	Phase 3 – BLA; Orphan Drug	TBD
plasminogen (human)	Kedrion	Ligneous conjunctivitis	Ophthalmic	Phase 3 – BLA; Orphan Drug	TBD
plinabulin	Beyondspring	NSCLC; Neutropenia/ leukopenia	IV	Phase 3 – NDA	TBD
pneumococcal 15-valent conjugate vaccine	Merck	Invasive pneumococcal disease prevention	IM	Phase 3 – NDA; Breakthrough Therapy	TBD
pollinex quattro grass	Allergy Therapeutics	Allergic rhinitis	SC	Phase 3 – BLA	TBD
ponesimod	Janssen	MS (relapsing)	Oral	Phase 3 – NDA	TBD
ranibizumab (biosimilar to Genentech's Lucentis®)	Santo	Wet AMD	Intraocular	Phase 3 – BLA	TBD
relugolix	Myovant	Endometriosis; Uterine fibroids; Prostate cancer	Oral	Phase 3 – NDA	TBD
remestemcel-L	Mesoblast	GVHD	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
reproxalap	Aldeyra	Congenital ichthyosis	Topical	Phase 3 – NDA; Orphan Drug	TBD
resmetirom	Madrigal	NASH	Oral	Phase 3 – NDA	TBD
respiratory syncitial virus (RSV) F nanoparticle vaccine	Novavax	RSV infection prevention	IM	Phase 3 – BLA; Fast Track	TBD
ridinilazole	Summit	*Clostridium difficile*-associated diarrhea/ infection	Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
risankizumab-rzaa (Skyrizi®)	Abbvie	CD; UC; PsA	SC	Phase 3 – sBLA; Orphan Drug	TBD
risdiplam	Roche	Spinal muscular atrophy	Oral	Phase 3 – NDA; Orphan Drug	TBD
risperidone long-acting	Laboratorios Farmacéuticos Rovi	Schizophrenia	IM	Phase 3 – 505(b)(2) NDA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Amgen	RA; CLL/SLL; NHL (indolent); ANCA associated vasculitis	IV, SC	Phase 3 – BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Archigen	RA; CLL/SLL; NHL (indolent); ANCA associated vasculitis	IV	Phase 3 – BLA	TBD
rituximab (biosimilar to Genentech's Rituxan) (Truxima)	Teva	RA; CLL/SLL; ANCA associated vasculitis	IV	Phase 3 – sBLA	TBD
rivipansel	Pfizer	Sickle cell disease-related vaso-occulsive crisis	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
rivoceranib	LSK Biopartners	Gastric cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
ropeginterferon alfa-2b	Pharmaessentia	Polycythemia vera	SC	Phase 3 – BLA; Orphan Drug	TBD
rovalpituzumab tesirine	Abbvie	SCLC	IV	Phase 3 – BLA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
roxadustat	AstraZeneca	Anemia due to CKD (dialysis-independent & dialysis-dependent); Anemia due to oncology treatment	Oral	Phase 3 – NDA	TBD
sacubitril/ valsartan (Entresto®)	Novartis	Heart failure (preserved ejection fraction)	Oral	Phase 3 – sNDA; Fast Track	TBD
satralizumab	Roche	Neuromyelitis optica (Devic's syndrome)	SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
savolitinib	AstraZeneca	RCC	Oral	Phase 3 – NDA	TBD
sci-B-Vac	VBI Vaccines	Hepatitis B (HBV) prevention	IM	Phase 3 – BLA	TBD
seladelpar	Cymabay	Primary biliary cholangitis/hepatic fibrosis	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
selinexor (Xpovio™)	Karyopharm	Sarcoma	Oral	Phase 3 – sNDA; Orphan Drug	TBD
selonsertib	Gilead	NASH	Oral	Phase 3 – NDA	TBD
semaglutide (Ozempic)	Novo Nordisk	Obesity	SC	Phase 3 – sNDA	TBD
serlopitant	Menlo	Pruritus associated with prurigo nodularis (PN)	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD
setmelanotide	Rhythm	Obesity related to leptin receptor deficiency or pro-opiomelanocortin deficiency	SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
sodium hyaluronate/ triamcinolone hexacetonide	Anika	Osteoarthritis (knee)	Intraarticular	Phase 3 – NDA	TBD
sodium oxybate (low-dose)	Jazz	Narcolepsy	Oral	Phase 3 – NDA	TBD
sodium oxybate (once-nightly dosing)	Avadel	Narcolepsy	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
sofpironium bromide	Bricknell	Hyperhidrosis	Topical	Phase 3 - NDA	TBD
somapacitan	Novo Nordisk	Growth hormone deficiency (adults)	SC	Phase 3 – BLA	TBD
somatrogon	Opko	Growth hormone deficiency	SC	Phase 3 – BLA; 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	UTI (uncomplicated)	IV, Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
sutimlimab	Sanofi	Cold agglutnin disease	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
suvodirsen	Wave Life Sciences	Duchenne muscular dystrophy	IV	Phase 3 – NDA; Orphan Drug	TBD
tabelecleucel	Atara	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
tadalafil film	Intelgenx/Aquestive	Erectile dysfunction	Oral	Phase 3 – 505(b)(2) NDA	TBD
tafasitamab	Morphosys	DLBCL	IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
talditercept alfa	Roche	Duchenne muscular dystrophy	SC	Phase 3 – BLA; Orphan Drug	TBD
tanezumab	Pfizer	Osteoarthritis; Chronic low back pain; Cancer pain	IV	Phase 3 – BLA; Fast Track	TBD
tasimelteon (Hetlioz)	Vanda	Smith-Magenis syndrome	Oral	Phase 3 – sNDA; Orphan Drug	TBD
tecarfarin	Espero	Atrial fibrillation	Oral	Phase 3 – NDA	TBD
tenapanor	Ardelyx	Hyperphosphatemia	Oral	Phase 3 – NDA	TBD
teprasiran	Quark	Delayed graft function; Acute renal injury prevention following cardiac surgery	IV	Phase 3 – NDA; Orphan Drug	TBD
terlipressin	Mallinckrodt	Hepatorenal syndrome	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
tezepelumab	AstraZeneca	Asthma	SC	Phase 3 – BLA; Breakthrough Therapy	TBD
timapiprant	Chiesi	Asthma	Oral	Phase 3 – NDA	TBD
timbetasin	Regenerx	Dry eye disease	Ophthalmic	Phase 3 – BLA; Orphan Drug	TBD
tirbanibulin	Almirall	Actinic keratoses	Oral	Phase 3 – NDA	TBD
tirzepatide	Eli Lilly	T1DM	SC	Phase 3 – NDA	TBD
tisagenlecleucel-t (Kymriah™)	Novartis	CLL/SLL	IV	Phase 3 – sBLA; Orphan Drug	TBD
tonogenchoncel-L	Kolon Tissuegene	Osteoarthritis (knee)	Intraarticular	Phase 3 – BLA	TBD
tralokinumab	AstraZeneca	Atopic dermatitis	SC	Phase 3 – BLA	TBD
tramadol	Fortress	Postsurgical pain	IV	Phase 3 – 505(b)(2) NDA	TBD
transcon PEG growth hormone	Ascendis	Growth hormone deficiency	SC	Phase 3 – BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Novartis	Breast cancer; Gastric/ gastroesophageal cancer	IV	Phase 3 – BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Tanvex	Breast cancer; Gastric/ gastroesophageal cancer	IV	Phase 3 – BLA	TBD
trastuzumab deruxtecan	Daiichi Sankyo	Breast cancer	IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
trastuzumab/pertuzumab	Genentech	Breast cancer	SC	Phase 3 – BLA	TBD
TRC101	Tricida	CKD	Oral	Phase 3 – NDA	TBD
treprostinil	Liquidia	PAH	Inhaled	Phase 3 – 505(b)(2) NDA	TBD
treprostinil (patch pump)	United Therapeutics	PAH	SC	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
ublituximab	TG	CLL/SLL; MS	IV	Phase 3 – BLA; Orphan Drug	TBD
ublituximab/umbralisib	TG	NHL (Indolent)	IV, Oral	Phase 3 – BLA; Orphan Drug	TBD
udenafil	Allergan	Erectile dysfunction	Oral	Phase 3 – NDA	TBD
umbralisib	TG	CLL/SLL; NHL (indolent); Marginal zone lymphoma	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
upadacitinib	Abbvie	Atopic dermatitis; Axial spondyloarthritis; PsA; CD; UC	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD
vadadustat	Akebia	Anemia due to CKD (dialysis-independent & dialysis-dependent)	Oral	Phase 3 – NDA	TBD
valoctocogene roxaparvovec	Biomarin	Hemophilia A	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
viaskin peanut	DBV	Peanut allergy (ages 4 to 11 years)	Transdermal	Phase 3 – BLA; Breakthrough Therapy; Fast Track	TBD
viloxazine	Supernus	ADHD	Oral	Phase 3 – NDA	TBD
visomitin	Mitotech	Dry eye disease	Ophthalmic	Phase 3 – NDA	TBD
vocimagene amiretrorepvec	Tocagen	Brain cancer	Intratumoral, IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
voclosporin	Aurinia	Lupus nephritis	Oral	Phase 3 – NDA; Fast Track	TBD
volanesorsen	Akcea	Lipodystrophy	SC	Phase 3 – NDA; Orphan Drug	TBD
vosoritide	Biomarin	Achondroplasia	SC	Phase 3 – NDA; Orphan Drug	TBD
voxelotor	Global Blood Therapeutics	Sickle cell disease-related idiopathic pulmonary fibrosis	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
vutrisiran	Alnylam	Transthyretin amyloid cardiomyopathy (ATTR-CM, wild type or hereditary); Transthyretin amyloid polyneuropathy	SC	Phase 3 - NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
zanubrutinib	Beigene	Waldenstrom macroglobulinemia; CLL/SLL	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
bupivacaine/meloxicam	Heron	Postsurgical pain	Instillation	CRL	TBD
celiprolol	Acer	Ehlers-Danlos syndrome (vascular)	Oral	CRL	TBD
dapagliflozin (Farxiga)	AstraZeneca	T1DM	Oral	CRL	TBD
fosfomycin	Nabriva	UTI (complicated)	IV	CRL	TBD
ketotifen	Bausch Health	Allergic conjunctivitis	Ophthalmic	CRL	TBD
mannitol	Pharmaxis	CF	Inhaled	CRL	TBD
olopatadine HCl/mometasone furoate	Glenmark	Allergic rhinitis	Intranasal	CRL	TBD
quizartinib	Daiichi Sankyo	AML (relapsed/refractory, FLT3-internal tandem duplication [ITD] mutation)	Oral	CRL	TBD
trigriluzole	Biohaven	Alzheimer's disease	Oral	CRL	TBD

GLOSSARY

ABSSSI Acute Bacterial Skin and Skin Structure Infection

ACR20 American College of Rheumatology 20% Improvement

ACR50 American College of Rheumatology 50% Improvement

ACR70 American College of Rheumatology 70% Improvement

ADHD Attention Deficit Hyperactivity Disorder

ALK Anaplastic Lymphoma Kinase

ALL Acute Lymphoblastic Leukemia

ALT Alanine Transaminase

AMD Age-Related Macular Degeneration

AML Acute Myeloid Leukemia

ANCA Antineutrophil Cytoplasmic Antibodies

ANDA Abbreviated New Drug Application

ARV Antiretroviral

ART Antiretroviral Therapy

AS Ankylosing Spondylitis

AST Aspartate Aminotransferase

BLA Biologics License Application

BsUFA Biosimilar User Fee Act

CAP Community Acquired Pneumonia

CABP Community Acquired Bacterial 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

CNS Central Nervous System

COPD Chronic Obstructive Pulmonary Disease

CRC Colorectal Cancer

CRL Complete Response Letter

CV Cardiovascular

CVD Cardiovascular Disease

DAS28-CRP Disease Activity Score-28 with C Reactive Protein

DEA Drug Enforcement Administration

DLBCL Diffuse Large B Cell Lymphoma

DMARD Disease Modifying Antirheumatic Drug

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

GVHD Graft Versus Host Disease

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

HSCT Hematopoietic Stem Cell Transplant

HTN Hypertension

HR Hazard Ratio

IBS Irritable Bowel Syndrome

IBS-C Irritable Bowel Syndrome, Constipation Predominant

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

MRSA Methicillin-Resistant *Staphylococcus Aureus*

MS Multiple Sclerosis

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

ODT Orally Disintegrating Tablet

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

PTSD Post-Traumatic Stress Disorder

Q Quarter

QIDP Qualified Infectious Diseases Product

QOL Quality of Life

RA Rheumatoid Arthritis

RBC Red Blood Cell

RCC Renal Cell Carcinoma

REMS Risk Evaluation and Mitigation Strategy

RMAT Regenerative Medicine Advanced Therapy

RTOR Real-Time Oncology Review

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 Inhibitor

SNRI Serotonin and Norepinephrine Reuptake Inhibitor

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

VEGF Vascular Endothelial Growth Factor

WBC White Blood Cell

WHO World Health Organization

XR Extended-Release

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