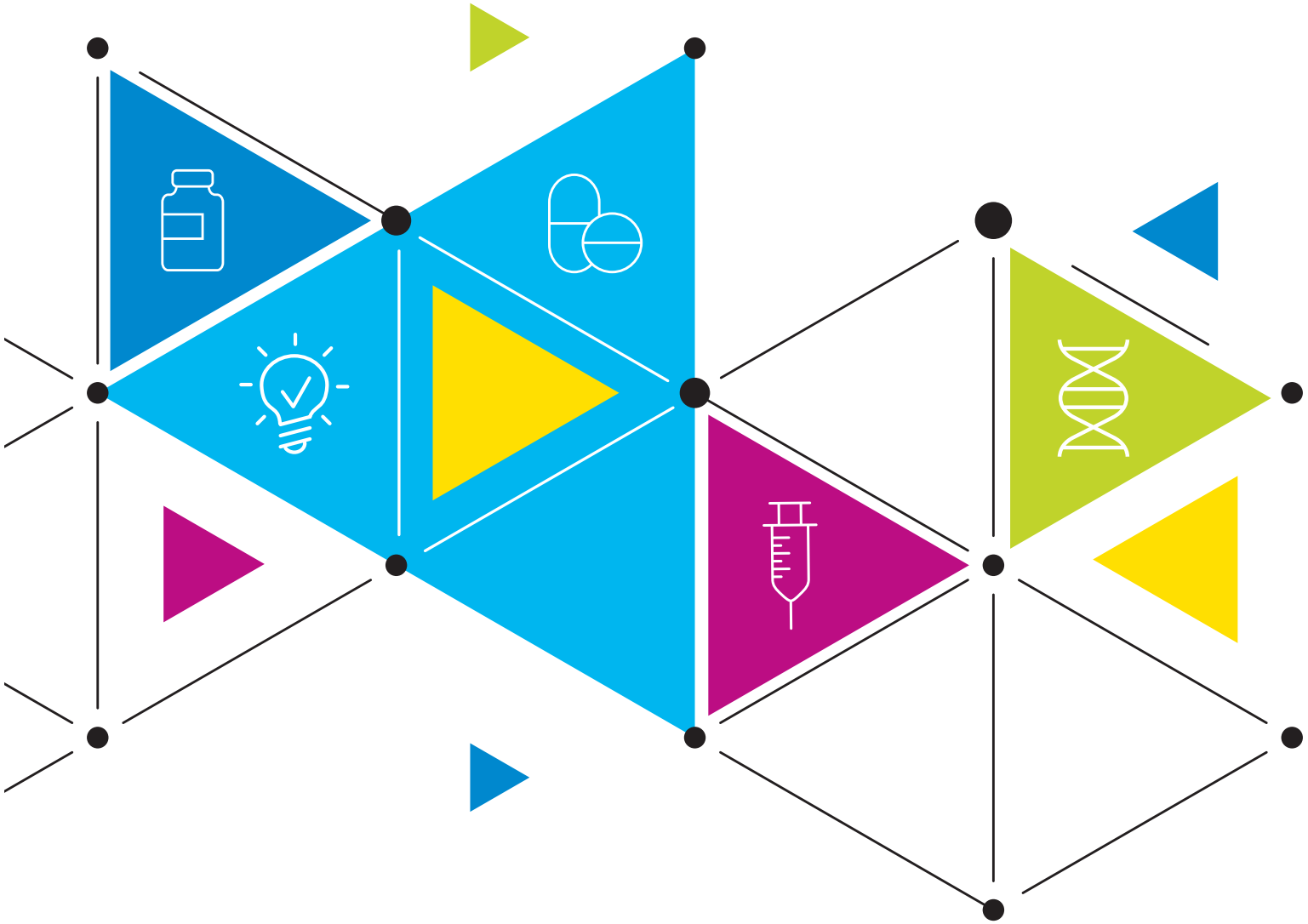
OCTOBER 2019

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.

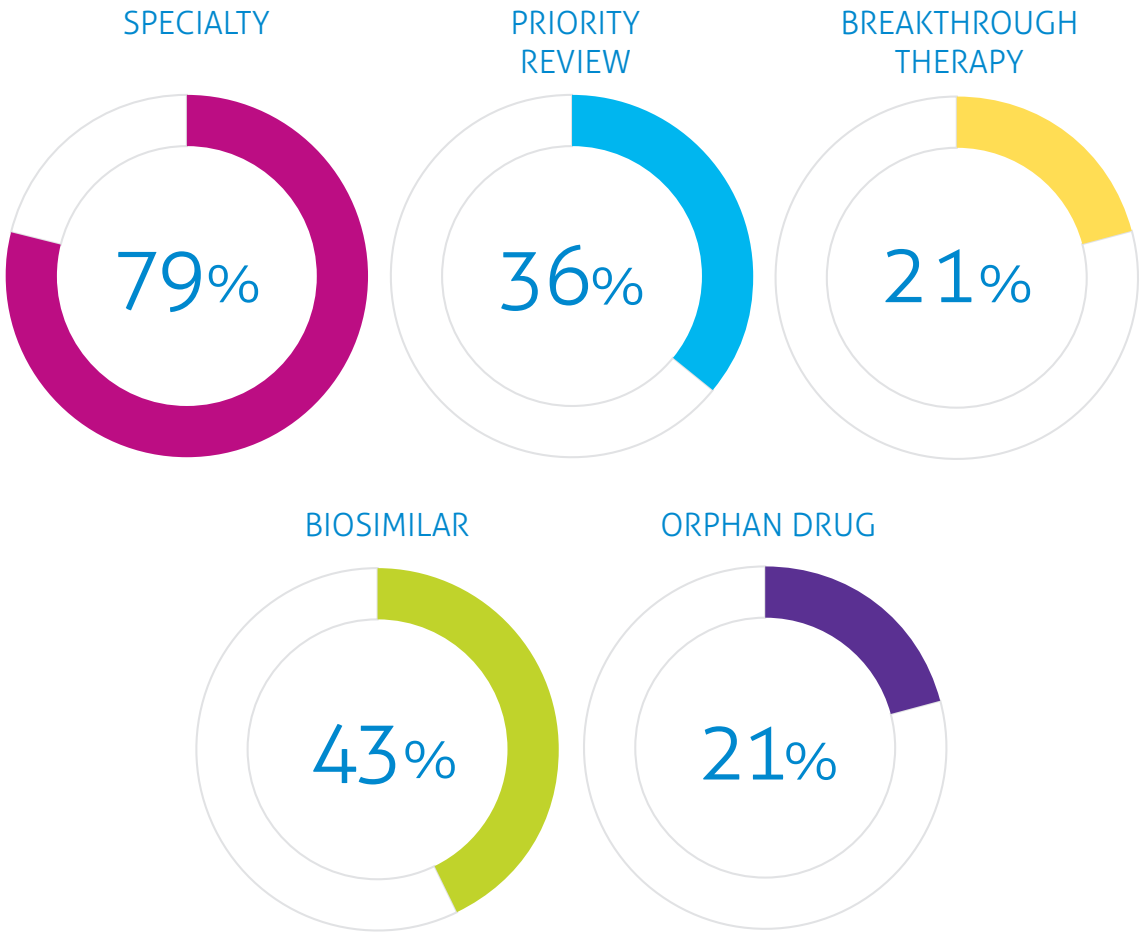
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year 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 FDA-approved 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. So far in 2019, the US FDA has approved 33 novel drugs. This count does not include approvals for several biologics or the record-breaking 1-time infusion gene therapy for select pediatric patients with spinal muscular atrophy. 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, cardiology, and investigational agents for peanut allergies. Moreover, gene therapy for hemophilia and sprouting products for ophthalmology, hematology, women's health, as well as oral options for postpartum depression 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.

Cardiology

bempedoic acid *oral*

Esperion

PROPOSED INDICATIONS

Treatment of elevated low-density lipoprotein cholesterol (LDL-C) despite the use of currently accessible therapies

CLINICAL OVERVIEW

Bempedoic acid blocks cholesterol production in the liver by inhibiting adenosine triphosphate (ATP) citrate lyase (ACL). Its action takes place upstream of statins in the cholesterol synthesis pathway.

Safety and efficacy of bempedoic acid were evaluated in the CLEAR program that included 4 placebo-controlled trials with patients (n=3,621 total) at high CV risk on maximally tolerated statin therapy (including 1 trial with statin-intolerant patients). Across the 4 studies, after 12 weeks of treatment with bempedoic acid, the LDL-C reduction from baseline ranged from 18% to 31%, and the reduction in high-sensitivity C-reactive protein (hsCRP), an inflammatory marker for CVD, ranged from 19% to 33%. Incidences of major adverse CV events (MACE) and new-onset or worsening diabetes were also lower with bempedoic acid compared to placebo. An additional study assessed bempedoic acid given in combination with ezetimibe. Enrollment included patients (n=382) with or at high risk for ASCVD who were on maximally tolerated statin therapy. At week 12, LDL-C lowering was significantly greater with the fixed-dose combination (36.2%) compared to either component alone (ezetimibe, 23.2%; bempedoic acid, 17.2%). Bempedoic acid was well tolerated. Myalgia and muscular weakness were reported at similar rates for bempedoic acid and placebo, whereas gout was reported more often in patients treated with bempedoic acid (CLEAR Harmony: 1.2% versus 0.3%; CLEAR Serenity: 1.7% versus 0.9%). In the CLEAR Harmony trial, 13 (0.9%) deaths were reported in the bempedoic acid arm and 2 (0.3%) were reported in the placebo arm; according to the manufacturer, none were attributed to the study drug.

Bempedoic acid was studied at oral doses of 180 mg once daily. The fixed-dose combination consisted of bempedoic acid 180 mg and ezetimibe 10 mg administered orally once daily.

PLACE IN THERAPY

In the US, an estimated 33.5% of adults have high LDL-C, of which about one-third are in control. High cholesterol leads to an increase in heart disease risk by approximately twofold. Statins are the cornerstone treatment for hyperlipidemia, but their use can be limited by resistance and intolerance. When target cholesterol levels are not achieved despite maximally tolerated statin therapy, ezetimibe or a proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor is added.

If approved, bempedoic acid will introduce another option for LDL-C lowering in patients who are not at goal or are difficult to manage. It will likely face strong competition from injectable drugs targeting PCSK9 (evolocumab [Repatha®], alirocumab [Praluent®]), including the late-phase small-interfering ribonucleic acid (siRNA) inclisiran (FDA submission anticipated in 2019), which have demonstrated superior reductions in LDL-C compared to bempedoic acid. Potential competitive pricing of the small molecule, its oral route of administration, and lack of myalgias (a known limiting factor for statins) may allow it to garner market share in the hyperlipidemia space. A trial evaluating the effects of bempedoic acid on incidence of MACE in statin-intolerant patients is ongoing; results are expected in the second half of 2022.

FDA APPROVAL TIMELINE

February 21, 2020 - bempedoic acid
February 26, 2020 - bempedoic acid/ezetimibe

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$44	$157	$310	$578

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

enfortumab vedotin *IV*

Astellas

PROPOSED INDICATIONS

Treatment of patients with locally advanced or metastatic urothelial cancer who have received a PD-1/PD-L1 inhibitor and who have received platinum-containing chemotherapy in the neoadjuvant/adjuvant, locally advanced, or metastatic setting

CLINICAL OVERVIEW

Enfortumab vedotin is an antibody-drug conjugate (ADC) directed at Nectin-4, an antigen expressed in various cancers, particularly bladder cancers (97%). The ADC represents a fusion of a fully human antibody with a microtubule-disrupting agent to deliver a highly cytotoxic medicine to the tumor site.

An ongoing, open-label, phase 2 trial evaluated enfortumab vedotin in patients (cohort 1, n=128) with the proposed indication noted above. A blinded independent review panel determined an ORR of 44%, CR of 12%, median DOR of 7.6 months, and median OS of 11.7 months. Response was seen as early as the first treatment cycle with similar rates regardless of number of prior therapies, liver metastases, and non-response to a PD-1/PD-L1 inhibitor. The most common treatment-related adverse events (≥ 40%) were fatigue, alopecia, decreased appetite, rash, and peripheral neuropathy. Treatment-related adverse events grade 3 or higher were neutropenia, anemia, and fatigue. A randomized phase 3 confirmatory trial is ongoing.

Enfortumab vedotin was administered via IV infusion on days 1, 8, and 15 of each 28-day cycle.

PLACE IN THERAPY

It is projected that 80,470 new cases and 17,670 deaths related to bladder cancer will occur in the US in 2019. Urothelial carcinoma accounts for over 90% of all bladder cancers, the majority of which are diagnosed in elderly men. Approximately 25% of patients present with muscle-invasive or metastatic disease and require radical cystectomy or transurethral resection of bladder tumors (TURBT) followed by concurrent radiation therapy and systemic chemotherapy. Long-term outcomes of first-line platinum-based treatment for locally advanced and metastatic urothelial carcinoma remain suboptimal, and nearly half of patients with advanced disease are ineligible for platinum-based therapy. PD-1/PD-L1 inhibitors (e.g., atezolizumab, avelumab, durvalumab, nivolumab, pembrolizumab) may be used second-line or as first-line in select platinum-ineligible patients. The fibroblast growth factor receptor (FGFR) inhibitor erdafitinib was approved in April 2019 for FGFR-positive tumors following progression despite platinum-based therapy.

Currently only a handful of ADCs are approved in the US, with an emerging pipeline focusing mostly on solid tumors. Dose-limiting toxicity has been a concern of ADCs, as evident by the voluntary withdrawal of gemtuzumab, which was later reintroduced with revised labeling.

If approved, the ADC enfortumab vedotin will be the first treatment for patients with advanced urothelial carcinoma who do not respond to PD-1/PD-L1 inhibitors after failure of platinum-based chemotherapy, in which roughly 80% of patients do not respond. Enrollment is ongoing for a phase 2 cohort in patients without prior platinum-containing chemotherapy. Furthermore, a trial is underway to evaluate enfortumab vedotin in earlier treatment lines, including in combination with pembrolizumab and/or platinum chemotherapy, in newly diagnosed patients, as well as patients who progressed from earlier-stage disease.

FDA APPROVAL TIMELINE

March 13, 2020

✓ Breakthrough Therapy ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$20	$80	$149	$220

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

Neurology

eptinezumab *IV*

Alder

PROPOSED INDICATIONS

Migraine prevention

CLINICAL OVERVIEW

Eptinezumab is a humanized monoclonal antibody that inhibits calcitonin gene-related peptide (CGRP).

The phase 3, double-blind PROMISE 1 (n=888) and PROMISE 2 (n=1,072) trials evaluated eptinezumab in patients with episodic migraine (EM) and chronic migraine (CM), respectively. At baseline, mean monthly migraine days (MMD) were 8.6 and 16.1 in the 2 studies, respectively. During months 1 through 3, significant reductions in mean MMD were demonstrated with eptinezumab 100 mg and 300 mg over placebo in both trials (PROMISE 1: 3.9, 4.3, and 3.2 days, respectively; PROMISE 2: 7.7, 8.2, and 5.6 days, respectively). When evaluated by migraine type, significantly more CM patients treated with either dose of eptinezumab achieved ≥ 75% reduction in MMD during months 1 through 3 compared to placebo-treated patients, and the difference was recorded as early as 1 month. In EM patients, a significant difference was only seen with the 300 mg dose compared to placebo. Reductions in MMD with eptinezumab relative to placebo were maintained through month 12 in CM patients; in EM patients, this was maintained through month 9 and to a smaller extent through month 12. Patient-reported reductions in migraine disability and acceptable tolerability with eptinezumab 300 mg were demonstrated in the open-label PREVAIL trial (n=128) for up to 2 years in patients with CM.

Both doses of eptinezumab were administered IV every 12 weeks.

PLACE IN THERAPY

Over 37 million Americans suffer from migraine headaches, the majority of whom are women. Migraine attacks can be debilitating, with pain lasting hours to days. If approved, eptinezumab will be the first IV-administered CGRP-targeting monoclonal antibody that is infused quarterly. To succeed in the marketplace, eptinezumab will need to distinguish itself from the existing self-administered SC agents for migraine prophylaxis that target CGRP. These include once-monthly erenumab (Aimovig®), fremanezumab (Ajovy®), and galcanezumab (Emgality®). Moreover, fremanezumab has an every-3-month dosing regimen, and galcanezumab carries an indication for cluster headache prevention.

FDA APPROVAL TIMELINE

February 21, 2020

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$48	$170	$341	$513

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

Metabolic

givosiran *SC*

Alnylam

PROPOSED INDICATIONS

Treatment of acute hepatic porphyria (AHP)

CLINICAL OVERVIEW

Givosiran is an siRNA that prevents the production of porphyrin and hemes by inhibiting translation and expression in the liver of amino levulinate synthase 1 (ALAS-1), a key protein in heme production.

The phase 3, double-blind ENVISION trial evaluated safety and efficacy of givosiran on the rate of AHP attacks in 94 patients with AHP (ages ≥ 12 years). Patients had a history of ≥ 2 attacks in the 6 months prior to the study. Compared to placebo, after 6 months of treatment, givosiran resulted in a 74% mean reduction and 90% median reduction in the annualized attack rate (AAR) measured by the composite of hospitalization, urgent healthcare visit, and hemin administration (median AAR of 1 with givosiran versus 10.7 with placebo). Response was seen as early as 1 month after starting therapy. Among patients treated with givosiran, 50% were attack-free during the 6 months treatment period versus 16% treated with placebo. Significant improvement in secondary endpoints were also reported; this included urinary aminolaevulinic acid (ALA) levels, porphobilinogen (PBG) levels, and days on hemin. While fewer days of opioid and non-opioid analgesia treatment were reported, the reduction in daily worst pain narrowly missed statistical significance (p=0.053). No significant effect on fatigue was observed. An ongoing open-label extension study (n=93) demonstrated a duration of response for up to 30 months. Liver enzyme elevation more than 3 times the upper limit of normal was reported more often with givosiran than with placebo (14.6% versus 2.2%) and led to treatment discontinuation in 1 patient on givosiran.

Givosiran was studied as a 2.5 mg/kg dose HCP-administered SC once monthly.

PLACE IN THERAPY

AHPs are a rare group of inherited metabolic disorders caused by a lack of key enzymes necessary for hemoglobulin production in the liver that is controlled by ALAS-1. This results in a build-up of neurotoxic intermediates, such as ALA and PBG. Acute attacks can be triggered by stress, infection, alcohol consumption, dietary fasting, hormone fluctuations, and certain medications. AHPs are often misdiagnosed due to the nonspecific nature of symptoms such as abdominal pain, nausea, and neurologic changes. Hospitalization or urgent medical care may be necessary for severe attacks. Current treatment of acute episodes focuses on pain relief (often requiring opioid use), increased glucose intake, and IV administration of hemin. Care is also required for complications including hypertension, chronic neuropathic pain, kidney disease, and liver disease. Extreme cases may require liver transplantation.

In clinical trials, HCP-administered givosiran resulted in a significant reduction in AHP attacks; however, data suggest a potential increased risk for liver injury with its use. If approved, givosiran will be the first agent approved in the US for the treatment of AHP.

FDA APPROVAL TIMELINE

February 4, 2020

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$6	$55	$116	$178	$225

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

ozanimod *oral*

Celgene

PROPOSED INDICATIONS

Treatment of relapsing forms of multiple sclerosis (RMS)

CLINICAL OVERVIEW

Ozanimod is a selective sphingosine-1-phosphate (S1P)-1 and -5 receptor modulator that may reduce inflammation and cell damage and inhibit neurologic deficits in patients with RMS.

Safety and efficacy of once-daily ozanimod 0.5 mg and 1 mg were compared to once-weekly IM interferon beta-1a (IFN β-1a) (Avonex®) in the double-blind, phase 3 SUNBEAM (n=1,346) and phase 2/3 RADIANCE (n=1,320) trials in adults with RMS. Significantly lower adjusted annualized relapse rates (ARR) after 12 (SUNBEAM) and 24 (RADIANCE) months of therapy were reported with ozanimod 0.5 mg and 1 mg compared to IFN β-1a (SUNBEAM ARR: 0.18 and 0.24 versus 0.35, respectively; RADIANCE ARR: 0.22 and 0.17 versus 0.28, respectively). Compared to IFN β-1a, at 12 and 24 months, the adjusted mean number of new or enlarging T2 lesions on MRI was reduced by 25% and 34% for ozanimod 0.5 mg and by 48% and 42% for ozanimod 1 mg, respectively, for both time points. Similarly, fewer gadolinium-enhancing (GdE) lesions and slower brain volume loss were reported with ozanimod. The incidences of serious infection and cardiac adverse events were low across all treatment groups, and no cases of progressive multifocal leukoencephalopathy (PML) were reported.

PLACE IN THERAPY

Nearly 1 million people in the US are living with MS, and the majority have relapsing forms of the condition. Several disease-modifying therapies (DMT) indicated to treat RMS aim to decrease relapse rate and slow the accumulation of MRI-detected brain lesions. In clinical trials, ozanimod demonstrated dose-dependent efficacy for RMS and was shown to be superior to IFN β-1a.

Other oral S1P modulators available in the US include fingolimod (Gilenya®) and siponimod (Mayzent®; approved March 2019). Both medications are by Novartis and indicated to treat adults with RMS; fingolimod is approved in patients as young as 10 years. Fingolimod and certain other MS agents have been associated with PML, a rare and life-threatening viral brain infection. To date, PML has not been reported with siponimod or ozanimod. While cardiac events were infrequent with ozanimod, it remains to be seen if the product labeling will carry the S1P classwide CV precautions. Furthermore, both siponimod and ozanimod are metabolized in the liver by CYP enzymes; while CYP2C9 genotyping is required with siponimod, this need has not been identified with ozanimod.

In late 2017, Celgene filed its first submission of ozanimod to the FDA for RMS; however, the FDA refused to review the application due to insufficient pharmacology data at that time. If approved based on more complete data, ozanimod will provide a third once-daily, oral option for the treatment of RMS in adults. Janssen is expected to submit its S1P modulator ponesimod to the FDA in 2019 for RMS. Further, fingolimod generics could present additional competition in the MS space. Unlike the other S1P modulators, ozanimod is also in phase 3 studies for ulcerative colitis (FDA submission expected in 2019) and Crohn's disease. Some experts speculate that these GI indications may necessitate a lower price point for ozanimod compared to other MS agents.

FDA APPROVAL TIMELINE

March 25, 2020

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$125	$348	$539	$662

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

Neurology

rimegepant *oral*

Biohaven

PROPOSED INDICATIONS

Acute treatment of migraine

CLINICAL OVERVIEW

Rimegepant is a calcitonin gene-related peptide (CGRP) receptor antagonist.

Three double-blind, randomized, placebo-controlled trials in adults with a history of migraine for ≥ 1 year (n=3,507) investigated the efficacy of rimegepant for the acute treatment of migraines. The pooled data revealed that among patients who experienced ≥ 4 migraine attacks per month (n=2,426), significantly more rimegepant-treated patients were free of migraine pain and free of the most bothersome symptom (MBS) 2 hours after the dose was administered compared to placebo-treated patients (pain-free: 20.6% versus 12.6%, respectively; MBS-free: 35.8% versus 26.9%, respectively). Greater pain-free and MBS-free rates were also reported with rimegepant in those suffering < 4 migraine attacks per month (pain-free: 18.8% versus 11.3% for placebo; MBS-free: 37.6% versus 26.1% for placebo). Rimegepant was generally well tolerated, including normal electrocardiograms, vital signs, and liver function tests.

Rimegepant was administered orally to treat a single migraine episode at a dose of one 75 mg tablet (2 studies) or 75 mg orally disintegrating tablet (ODT) (1 study) at the onset of the attack. Effectiveness of the ODT dosage form was consistent with the tablet formulation at 2 hours post-dose.

PLACE IN THERAPY

If approved, rimegepant (tablet and ODT) is expected to be the second oral CGRP receptor antagonist for acute treatment of migraine episodes, following the potential approval for ubrogepant anticipated in December 2019. Both agents will likely compete with triptans for acute migraine treatment. Injectable CGRP antagonists (erenumab [Aimovig], fremanezumab [Ajovy], galcanezumab [Emgality]) are currently available in the US for *prevention* of migraine attacks. Response was seen with the rimegepant ODT formulation as early as 15 minutes post-dose, a measure that was not reported with ubrogepant tablet. Furthermore, the ODT formulation could be a distinguishing factor for rimegepant and an option for patients experiencing nausea related to migraine. While the development of 2 other oral CGRP antagonists were suspended due to liver toxicity, to date, neither rimegepant nor ubrogepant have been associated with increased liver enzymes.

Rimegepant is also being evaluated for migraine prevention in a phase 2/3 clinical trial and refractory trigeminal neuralgia in a phase 2 trial. Other agents that target CGRP in late phase trials include the oral CGRP receptor antagonist atogepant (dosed once or twice daily) for migraine prophylaxis and intranasal vazegepant for acute migraine treatment.

FDA APPROVAL TIMELINE

Late February 2020

✓ Priority Review (rimegepant ODT only)

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$58	$186	$409	$648

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

Oncology

tazemetostat *oral*

Epizyme

PROPOSED INDICATIONS

Metastatic or locally advanced epithelioid sarcoma

CLINICAL OVERVIEW

Tazemetostat selectively inhibits wild-type and mutated forms of enhancer of zeste homolog 2 (EZH2), and thereby prevents histone methylation-related cancer cell proliferation.

Submission to the FDA for approval of tazemetostat is supported by an ongoing, phase 2 trial in patients (n=62) with metastatic or locally advanced epithelioid sarcoma not eligible for curative surgery, including treatment-naïve and relapsed/refractory patients. At the time of data cut-off (September 2018), interim data revealed tazemetostat was associated with a 15% ORR and a 26% disease control rate (DCR). The median DOR had not yet been reached and OS was 82.4 weeks. Treatment-related adverse events were generally mild to moderate with fatigue, nausea, and cancer pain reported most often. Adverse events grade 3 or higher in severity were reported in 16% of patients, which included anemia and weight loss.

Tazemetostat was studied as a dose of 800 mg administered orally twice daily.

PLACE IN THERAPY

Epithelial sarcoma is a rare, high-grade, soft tissue tumor of the distal portions of the limbs. Most often diagnosed in young adult males, it accounts for < 1% of all soft tissue sarcomas. Epithelial sarcoma is typically slow growing and often mistaken as a benign process. Surgical resection is the mainstay of treatment; however, recurrence and metastasis is common and severe cases may necessitate partial limb amputation. Adjunctive therapy with radiation or chemotherapy (doxorubicin, ifosafamide) may provide limited benefit. EZH2 is overexpressed or mutated in a variety of cancer cells and plays a key role in tumor cell proliferation. Over 90% of epithelial sarcomas do not express integrase interactor 1 (INI1); loss of INI1 function allows for EZH2-driven tumor cell proliferation. Metastatic disease is associated with poor prognosis (median survival reportedly as low as 1 year).

If approved, tazemetostat will be the first oral targeted therapy for epithelial sarcoma and could replace chemotherapy in the vast majority of patients with INI1-negative tumors. While an Accelerated Review is expected by the FDA, a confirmatory trial is planned to further support an indication for epithelial sarcoma. Other cohorts of the ongoing phase 2 solid tumor trial are comprised of patients with other tumor types, such as malignant rhabdoid tumors and synovial sarcoma. Tazemetostat is also in phase 2 trials for lymphoma (e.g., DLBCL, NHL), mesothelioma, and select solid tumors.

FDA APPROVAL TIMELINE

January 23, 2020

✓ Orphan Drug ✓ Priority Review/Accelerated Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$2	$3	$5	$6

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

voxelotor *oral*

Global Blood Therapeutics

PROPOSED INDICATIONS

Sickle cell disease (SCD)

CLINICAL OVERVIEW

Voxelotor increases hemoglobin's (Hb) affinity to oxygen and inhibits sickle Hb (HbS) polymerization. This leads to a reduction in distortion and aggregation of RBCs.

The double-blind, phase 3 HOPE study evaluated 2 doses of voxelotor (once-daily 1,500 mg and 900 mg) in 274 patients ages ≥ 12 years with SCD. Approximately 75% of patients were taking hydroxyurea (HU). At 24 weeks, in the intent-to-treat analysis, a Hb response (> 1g/dL Hb increase from baseline) was reported in 51% of patients treated with voxelotor 1,500 mg and 7% of those who received placebo; response occurred regardless of HU use. In addition, at week 24, treatment with voxelotor 1,500 mg led to fewer acute anemic episodes compared to placebo (annualized rate, 0.06 and 0.18, respectively) and significantly greater improvements in Hb, indirect bilirubin, and percentage of reticulocytes. A trend toward reduced vaso-occlusive crisis (VOC) incidence was observed, but the differences between voxelotor and placebo were not significant (annualized adjusted rate: 2.77 for voxelotor 1,500 mg, 3.19 for placebo). While improvements were reported with voxelotor 900 mg, statistical significance was not reported for any measure according to the prespecified hierarchical study design. The most common adverse effects reported with voxelotor (≥ 20%) were headache and diarrhea, most of which were of grade 1 or 2 severity.

PLACE IN THERAPY

An estimated 100,000 people in the US are diagnosed with SCD, with the highest prevalence in African Americans. The hereditary condition is characterized by sticky, sickle-shaped RBCs. The abnormal RBCs cause chronic anemia, vaso-occlusion, and eventual multiorgan damage. People with SCD have a reduced life expectancy by approximately 30 years.

Currently, oral HU with or without L-glutamine (Endari®) is the only approved pharmacologic treatment for SCD in the US. It is indicated to reduce the need for blood transfusion in patients with recurrent VOCs and is associated with leukemogenic and carcinogenic toxicities. While allogeneic bone marrow transplantation may be curative, its use is limited by associated risks and lack of matched donors. Novartis' IV monoclonal antibody crizanlizumab targets P-selectin mediated multi-cellular adhesion and has been submitted to the FDA for the prevention of SCD-related VOCs; approval is anticipated in January 2020.

If approved, oral voxelotor could be a first-in-class disease-modifying agent for adults and adolescents with SCD; however, its clinical impact on SCD remains unclear. While voxelotor reduces hemolysis and anemia in patients with SCD, it did not demonstrate statistically significant improvement on the incidence of VOC, a notable manifestation of SCD with negative impact on survival. Moreover, while stable erythropoietin levels observed in clinical trials suggest no negative effect on oxygen delivery, questions remain regarding voxelotor's effect on tissue oxygenation, particularly in the brain. Additional research, including a transcranial Doppler study, may provide answers and expand the patient population (ages 2 to 11 years) for its use.

FDA APPROVAL TIMELINE

February 26, 2020

✓ Breakthrough Therapy ✓ Fast Track ✓ Orphan Drug ✓ Priority Review
✓ Rare Pediatric Disease

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$74	$309	$661	$1,062

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 2 landmark 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 4 key strategies: (1) improving biosimilar development and approval process; (2) maximizing scientific and regulatory clarity for sponsors; (3) effective communications for patients, clinicians, and payers; and (4) reducing unfair tactics that may delay market approval and entry. The BAP strives to promote access to biosimilar products and reduce healthcare costs.

To date, a total of 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 groups 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 are paving the way for increased 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
$14,693	$15,584	$16,213	$16,676	$13,352

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

Blood Modifier
filgrastim *IV, SC*

Apotex and Kashiv 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

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$195	$165	$145	$131	$120

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 the 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

December 14, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$ 2,915	$2,334	$1,925	$1,606	$1,304

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 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/Sandoz (Ziextenzo)
Pending

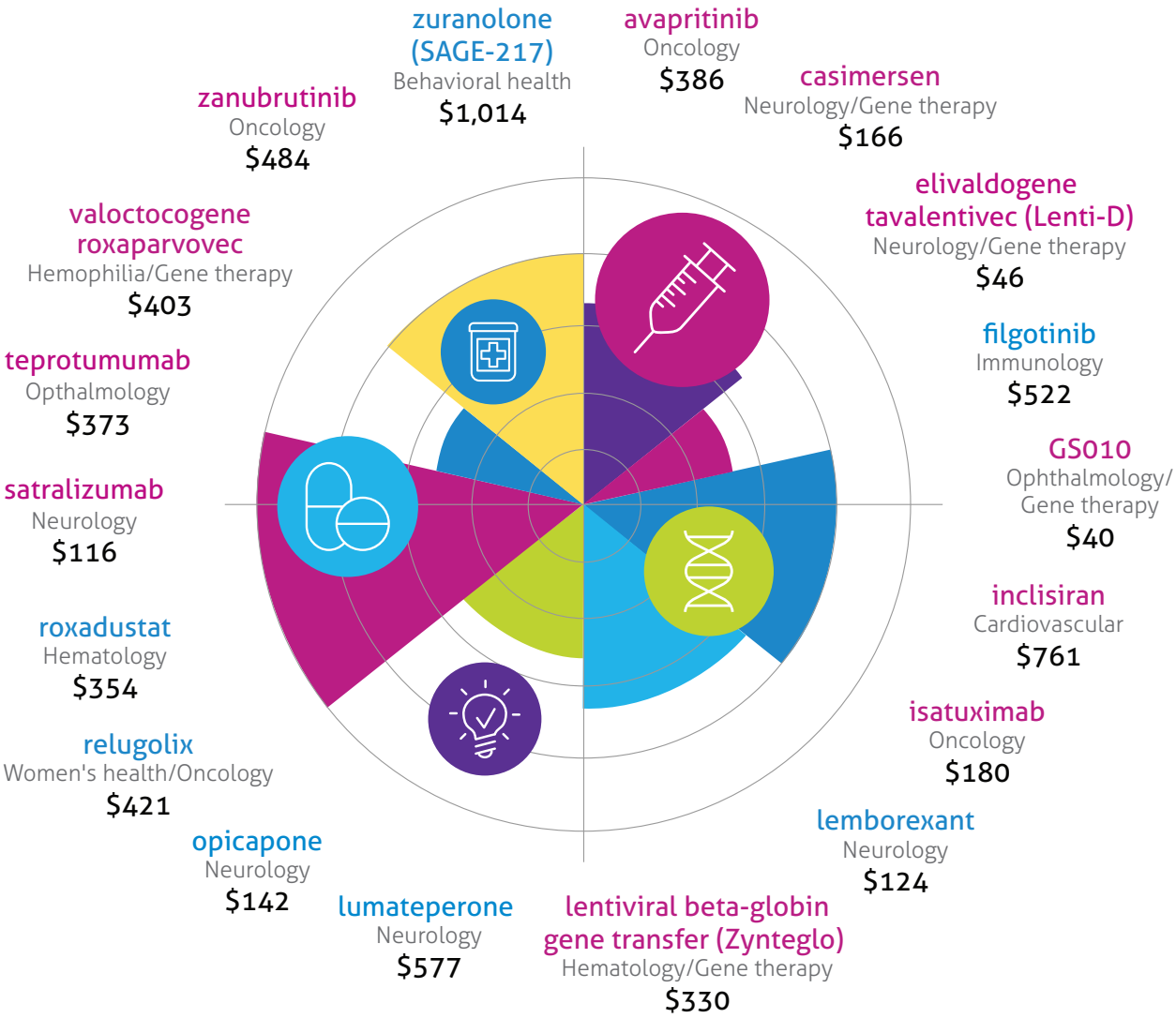
FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$3,021	$2,456	$2,073	$1,776	$1,560

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.*

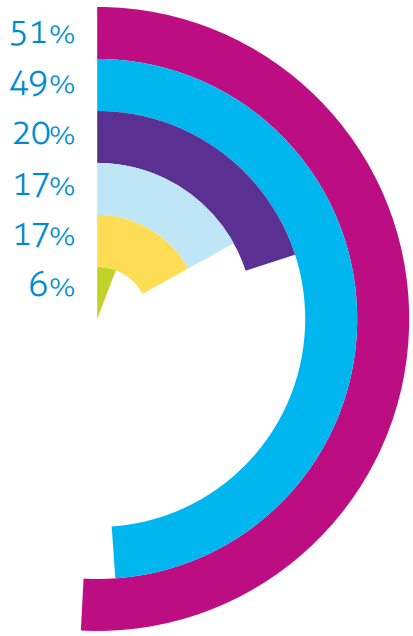


★ Specialty drug names appear in magenta throughout the publication.

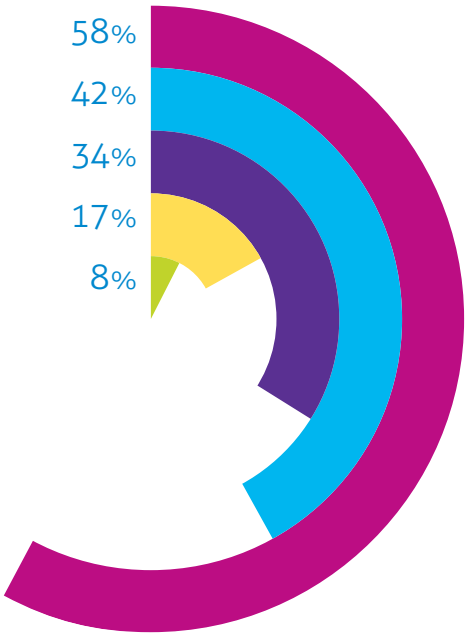
Pipeline Drug List

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
lorcaserin (Belviq®)	Eisai	Obesity (long-term use)	Oral	Submitted – sNDA	Oct-Nov 2019
lorcaserin ER (Belviq XR®)	Eisai	Obesity (long-term use)	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
influenza vaccine quadrivalent (Fluzone®) high-dose	Sanofi	Influenza prevention (ages ≥ 65 years)	IM	Submitted – sBLA	Oct-Dec 2019
naloxone single-dose prefilled syringe	Adamis	Substance use disorder	IM	Submitted – 505(b)(2) NDA	10/31/2019
dapagliflozin (Farxiga®)	AstraZeneca	T2DM CV outcomes	Oral	Submitted – sNDA	Nov-Dec 2019
RVT-802 (postnatal thymus tissue transplant)	Enzyvant	Pediatric congenital athymia	TBD	Submitted – BLA; Breakthrough Therapy; Orphan Drug; Priority Review; Rare Pediatric Disease; RMAT	Nov-Dec 2019
dulaglutide (Trulicity®)	Eli Lilly	T2DM CV outcomes	SC	Submitted – sNDA	Nov 2019 - Jan 2020
rifabutin/amoxicillin/ omeprazole	Redhill	*Helicobacter pylori* infection	Oral	Submitted – 505(b)(2) NDA; Fast Track; Priority Review; QIDP	11/01/2019
testosterone undecanoate	Lipocine	Hypogonadism	Oral	Submitted – 505(b)(2) NDA	11/09/2019
cefiderocol	Shionogi	UTI (complicated)	IV	Submitted – NDA	11/14/2019
ethinyl estradiol/ levonorgestrel	Agile	Contraception	Transdermal	Submitted – 505(b)(2) NDA	11/15/2019
cenobamate	SK Biopharmaceuticals	Partial-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
methotrexate	Cumberland	PSO	SC	Submitted – NDA	December 2019
ubrogepant	Allergan	Migraine treatment (adults)	Oral	Submitted – NDA	December 2019
vedolizumab (Entyvio®)	Takeda	UC	SC	Submitted – sBLA	Dec 2019 - Jan 2020
atezolizumab (Tecentriq®)	Genentech	NSCLC (non-squamous, 1st-line, with nab-paclitaxel)	IV	Submitted – sBLA	12/02/2019
luspatercept	Acceleron	Beta-thalassemia	SC	Submitted – BLA; Fast Track; Orphan Drug; Priority Review	12/04/2019
infliximab (biosimilar to Janssen's Remicade)	Amgen	RA; AS; PSO; PsA; CD; UC	IV	Submitted – BLA	12/14/2019
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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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
icosapent ethyl (Vascepa®)	Amarin	Major CV event risk reduction	Oral	Submitted – sNDA; Priority Review	12/27/2019
lemborexant	Eisai	Insomnia	Oral	Submitted – NDA	12/27/2019
lumateperone	Intra-cellular Therapies	Schizophrenia	Oral	Submitted – NDA; Fast Track	12/27/2019
crizanlizumab	Novartis	Sickle cell disease-related vaso-occlusive crisis	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug; Priority Review	January 2020
peanut protein capsule (AR101)	Aimmune	Peanut allergy (children and adolescents)	Oral	Submitted – BLA; Breakthrough Therapy; Fast Track	January 2020
empagliflozin/linagliptin/ metformin ER	Boehringer Ingelheim	T2DM	Oral	Submitted – NDA	Jan-Mar 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 (Ozempic®)	Novo Nordisk	T2DM-related CV risk reduction	SC	Submitted – sNDA	01/20/2020
semaglutide (Rybelsus™)	Novo Nordisk	T2DM-related CV risk reduction	Oral	Submitted – sNDA	01/20/2020
tazemetostat	Epizyme	Epithelial sarcoma	Oral	Submitted – NDA; Orphan Drug; Priority Review	01/23/2020
fidaxomicin (Dificid®)	Merck	*Clostridium difficile*-associated diarrhea/ infection (ages ≥ 6 months)	Oral	Submitted – sNDA; Fast Track; Orphan Drug	01/24/2020
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
rimegepant	Biohaven	Migraine treatment	Oral	Submitted – NDA; Priority Review (ODT only)	Late February 2020
empagliflozin (Jardiance®)	Boehringer Ingelheim	T1DM	Oral	Submitted – sNDA	Feb-Mar 2020
inebilizumab	Viela	Neuromyelitis optica (Devic's syndrome)	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug	Feb-Mar 2020
paclitaxel injection concentrate for suspension	Sun	Breast cancer	IV	Submitted – 505(b)(2) NDA	Feb-Mar 2020
givosiran	Alnylam	Acute hepatic porphyria	SC	Submitted – NDA; Breakthrough Therapy; Orphan Drug; Priority Review	02/04/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
avapritinib	Blueprint	Gastrointestinal stromal tumor (PDGFRA exon 18 mutant)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review; RTOR	02/14/2020
pembrolizumab (Keytruda®) - 6 week dosing regimen	Merck	Classical Hodgkin lymphoma; Gastric cancer; HCC, Melanoma; Merkel cell carcinoma; Primary mediastinal large B cell lymphoma	IV	Submitted – sBLA; Breakthrough Therapy; Orphan Drug	02/18/2020
bempedoic acid	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted – NDA	02/21/2020
eptinezumab	Alder	Migraine prevention	IV	Submitted – BLA	02/21/2020
amisulpride	Acacia	Post-operative nausea/ vomiting	IV	Submitted – NDA	02/26/2020
bempedoic acid/ezetimibe	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted – NDA	02/26/2020
voxelotor	Global Blood Therapeutics	Sickle cell disease	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review; Rare Pediatric Disease	02/26/2020
zanubrutinib	Beigene	Mantle cell lymphoma	Oral	Submitted – NDA; Breakthrough Therapy; Priority Review	02/27/2020
naloxone nasal spray	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 delayed-release oral granule (Procysbi®)	Horizon	Nephropathic cystinosis	Oral	Submitted – sNDA; Orphan Drug	Mar-Apr 2020
teprotumumab	Horizon	Graves' ophthalmopathy/ orbitopathy	IV	Submitted – BLA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	03/06/2020
exenatide SC pump	Intarcia	T2DM	SC	Submitted – NDA	03/09/2020
ebola vaccine	Merck	Ebola infection prevention	IM	Submitted – BLA; Breakthrough Therapy; Priority Review	03/13/2020
enfortumab vedotin	Astellas	Bladder cancer	IV	Submitted – BLA; Breakthrough Therapy; Priority Review	03/13/2020
lamotrigine oral liquid	Eton	Partial seizures; Primary generalized tonic-clonic seizures; Lennox-Gastaut syndrome	Oral	Submitted – 505(b)(2) NDA	03/17/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ozanimod	Celgene	MS (relapsing)	Oral	Submitted – NDA	03/25/2020
rizatriptan film	Gensco	Migraine treatment	Oral transmucosal	Submitted – 505(b)(2) NDA	03/26/2020
ferric pyrophosphate (Triferic®)	Rockwell	Anemia due to CKD (dialysis-dependent)	IV	Submitted – sNDA	03/27/2020
apremilast (Otezla®) - once daily	Celgene	PSO (scalp)	Oral	Submitted – sNDA	April 2020
trastuzumab deruxtecan	Daiichi Sankyo	Breast cancer (HER2+, metastatic, with capecitabine, 3rd-line)	IV	Submitted – BLA; Breakthrough Therapy; Fast Track; Priority Review	April 2020
bupivacaine/meloxicam	Heron	Postsurgical pain	Instillation	Submitted – NDA; Breakthrough Therapy; Fast Track	04/01/2020
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 meningitis prevention	IM	Submitted – BLA	04/24/2020
opicapone	Neurocrine Biosciences	Parkinson's disease ("off" episodes)	Oral	Submitted – NDA	04/24/2020
treprostinil (patch pump)	United Therapeutics	PAH	SC	Submitted – 505(b)(2) NDA; Orphan Drug	04/27/2020
isatuximab	Sanofi	Multiple myeloma	IV	Submitted – BLA; Orphan Drug	04/30/2020
ramucirumab (Cyramza®)	Eli Lilly	NSCLC (EGFR+, 1st-line)	IV	Submitted – sBLA	May 2020
neratinib (Nerlynx®)	Puma	Breast cancer (HER2+, metastatic, with capecitabine, 3rd-line)	Oral	Submitted – sNDA	05/01/2020
veverimer	Tricida	CKD-related metabolic acidosis	Oral	Submitted – NDA	05/04/2020
dasotraline	Sumitomo Dainippon	Binge eating disorder	Oral	Submitted – NDA	05/14/2020
obeticholic acid (Ocaliva®)	Intercept	NASH	Oral	Submitted – sNDA; Breakthrough Therapy	05/27/2020
enzalutamide (Xtandi®)	Astellas	Prostate cancer (metastatic, hormone-sensitive)	Oral	Submitted – sNDA; Fast Track; Priority Review	05/30/2020
abicipar pegol	Allergan	Wet AMD	Intraocular	Submitted – BLA	Jun-Jul 2020
viltolarsen	Nippon Shinyaku	Duchenne muscular dystrophy	IV	Submitted – NDA; Fast Track; Orphan Drug	06/02/2020
elagolix (Orilissa®)	Abbvie	Uterine fibroids	Oral	Submitted – NDA	06/05/2020
minocycline 1.5%	Foamix	Rosacea	Topical	Submitted – 505(b)(2) NDA	06/05/2020
nintedanib (Ofev®)	Boehringer Ingelheim	Pulmonary fibrosis	Oral	Submitted – sNDA; Breakthrough Therapy	July 2020
daratumumab (Darzalex®)	Janssen	Multiple myeloma	SC	Submitted – sBLA	07/10/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
guselkumab (Tremfya®)	Janssen	PsA	SC	Submitted – sBLA	07/16/2020
oxymetazoline 0.1% solution	Osmotica	Acquired blepharoptosis	Topical	Submitted – NDA	07/17/2020
calcipotriene/ betamethasone dipropionate	MC2	PSO	Topical	Submitted – 505(b)(2) NDA	07/24/2020
fenfluramine (low dose)	Zogenix	Dravet syndrome	Oral	Submitted – NDA; Fast Track; Orphan Drug	07/24/2020
fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®)	GlaxoSmithKline	Asthma (adults)	Inhaled	Submitted – sNDA	07/31/2020
triheptanoin	Ultrenyx	Fatty acid oxidation disorders	Oral	Submitted – NDA; Fast Track; Orphan Drug	07/31/2020
esketamine (Spravato®)	Janssen	MDD (with suicidal ideation with intent)	Intranasal	Submitted – sNDA; Breakthrough Therapy; Fast Track	08/02/2020
viaskin peanut	DBV	Peanut allergy (ages 4 to 11 years)	Transdermal	Submitted – BLA; Breakthrough Therapy; Fast Track	08/05/2020
ustekinumab (Stelara®)	Janssen	PSO (ages 6-11 years)	IV, SC	Submitted – sBLA	08/07/2020
dolutegravir/lamivudine (Dovato®)	Viiv	HIV-1 infection (switch treatment in virologically suppressed adults)	Oral	Submitted – sNDA	08/16/2020
clascoterone	Cassiopea	Acne	Topical	Submitted – NDA	08/20/2020
cantharidin	Verrica	Molluscum contagiosum	Topical	Submitted – NDA	09/16/2020
somapacitan	Novo Nordisk	Growth hormone deficiency (adults)	SC	Submitted – BLA	09/21/2020
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
pegfilgrastim (biosimilar to Amgen's Neulasta)	Novartis/Sandoz	Neutropenia/leukopenia	SC	Submitted – BLA	Pending
5-aminolevulinic acid	Biofrontera	Actinic keratoses (with conventional photodynamic therapy)	Topical	Phase 3 – sNDA	TBD
abaloparatide-TD	Radius Health	Osteoporosis/osteopenia	Transdermal	Phase 3 – NDA	TBD
abametapir	Dr. Reddy's	Head lice (ages ≥ 6 months)	Topical	Phase 3 – NDA	TBD
abrocitinib	Pfizer	Atopic dermatitis	Oral	Phase 3 – NDA; Breakthrough Therapy	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
adalimumab (biosimilar to Abbvie's Humira)	Momenta	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 – BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Mylan/Biocon	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 – BLA	TBD
adeno-associated viral vector containing human coagulation factor IX	Uniqure	Hemophilia B	IV	Phase 3 – BLA; Breakthrough Therapy	TBD
ado-trastuzumab emtansine (Kadcyla®)	Genentech	Breast cancer (HER2+, adjuvant, with pertuzumab)	IV	Phase 3 – sBLA; Breakthrough Therapy	TBD
aducanumab	Biogen	Alzheimer's disease	IV	Phase 3 – BLA; Fast Track	TBD
aflibercept (biosimilar to Regeneron's Eylea®)	Mylan	Diabetic macular edema	Intraocular	Phase 3 – sBLA	TBD
albuterol (ProAir RespiClick®)	Teva	COPD	Inhaled	Phase 3 – sNDA	TBD
albutrepenonacog alfa (Idelvion®)	CSL	Hemophilia B (21-day dosing schedule)	IV	Phase 3 – sBLA; Orphan Drug	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, SC	Phase 3 – BLA; Fast Track	TBD
apalutamide (Erleada®)	Janssen	Prostate cancer (localized, metastatic, castration-resistant)	Oral	Phase 3 – sNDA	TBD
arimoclomol citrate	Orphazyme	Niemann-Pick disease	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
artesunate	La Jolla	Malaria (severe)	Not specified	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Orphan Drug	TBD
asciminib	Novartis	Chronic myelogenous leukemia	Oral	Phase 3 – NDA; Orphan Drug	TBD
ataluren (Translarna®)	PTC Therapeutics	Duchenne muscular dystrophy	Oral	Phase 3 – sNDA; Fast Track; Orphan Drug	TBD
atezolizumab (Tecentriq)	Genentech	CRC; HCC; RCC; Melanoma; Ovarian cancer	IV	Phase 3 – sBLA; Breakthrough Therapy; Orphan Drug	TBD
autologous genetically modified human dermal fibroblasts	Castle Creek	Epidermolysis bullosa	Intradermal	Phase 3 – BLA; Fast Track; Orphan Drug; RMAT	TBD
avacopan	Chemocentryx	ANCA-associated vasculitis	Oral	Phase 3 – NDA; Orphan Drug	TBD
avalglucosidase alfa	Sanofi	Pompe disease	IV	Phase 3 – BLA	TBD
azacitidine	Celgene	AML	Oral	Phase 3 – NDA	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
BCX7353	Biocryst	Hereditary angioedema	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
bedaquiline (Situro®)	Janssen	Tuberculosis (pediatric patients with pulmonary multidrug-resistant tuberculosis [MDR-TB])	Oral	Phase 3 – sNDA; Fast Track; Orphan Drug	TBD
benralizumab (Fasenra®)	AstraZeneca	Nasal polyposis	SC	Phase 3 – sBLA	TBD
benzoyl peroxide	Sol-gel	Rosacea	Topical	Phase 3 – 505(b)(2) NDA	TBD
benzoyl peroxide/tretinoin	Bausch Health	Acne	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
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 Kirin	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 – BLA	TBD
bexagliflozin	Theracos	T2DM	Oral	Phase 3 – NDA	TBD
biotin (high dose)	Medday	MS	Oral	Phase 3 – NDA	TBD
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease; Bipolar disorder	Oral	Phase 3 – sNDA; Fast Track	TBD
budenoside	Calliditas	Immunoglobulin A (IgA) nephropathy (Berger's disease)	Oral	Phase 3 – NDA; Orphan Drug	TBD
budesonide (viscous suspension)	Takeda	Esophagitis	Topical	Phase 3 – 505(b)(2) NDA; Breakthrough Therapy; Orphan Drug	TBD
budesonide MDI	AstraZeneca	COPD	Inhaled	Phase 3 – 505(b)(2) NDA	TBD
budesonide/albuterol	AstraZeneca	Asthma	Inhaled	Phase 3 – NDA	TBD
budesonide/formoterol MDI (Aerosphere delivery)	AstraZeneca	COPD	Inhaled	Phase 3 – NDA	TBD
cabotegravir (long-acting)	GlaxoSmithKline	HIV-1 infection pre-exposure prevention (PrEP)	IM	Phase 3 – NDA	TBD
cabozantinib (Cabometyx®)	Exelixis	RCC (1st-line, with nivoluab)	Oral	Phase 3 – sNDA; Breakthrough Therapy; Fast Track	TBD
calmangafodipir	Pledpharma	Chemotherapy-induced peripheral neuropathy	IV	Phase 3 – NDA	TBD
cannabidiol (Epidiolex®)	GW	Tuberous sclerosis complex; Rett syndrome	Oral	Phase 3 – sNDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
capmatinib	Novartis	NSCLC	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
capsaicin	Centrexion	Osteoarthritis	Intraarticular	Phase 3 – NDA; Fast Track	TBD
carbidopa/levodopa	Amneal	Parkinson's disease	Oral	Phase 3 – NDA	TBD
carglumic acid	Recordati	Hyperammonaemia secondary to autosomal genetic disorder	Oral	Phase 3 – NDA; Orphan Drug	TBD
casimersen	Sarepta	Duchenne muscular dystrophy	IV	Phase 3 – NDA; Orphan Drug	TBD
cedazuridine/decitabine	Otsuka	AML; Chronic myelomonocytic leukemia; Myelodysplastic syndrome	Oral	Phase 3 – NDA; Orphan Drug	TBD
cediranib	AstraZeneca	Ovarian cancer; Biliary tract cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
cefiderocol	Shionogi	HAP	IV	Phase 3 – NDA	TBD
ceftobiprole medocaril	Basilea	Skin and skin-structure infections (antibacterial)	IV	Phase 3 – NDA; Fast Track; QIDP	TBD
cenicriviroc mesylate	Allergan	NASH	Oral	Phase 3 – NDA; Fast Track	TBD
ceritinib (Zykadia®)	Novartis	NSCLC (ALK+, brain metastases)	Oral	Phase 3 – sNDA; Breakthrough Therapy; Orphan Drug	TBD
CM-AT	Curemark	Autism spectrum disorders	Oral	Phase 3 – BLA; Fast Track	TBD
colesevalam	Ironwood	Gastroesophageal reflux disease	Oral	Phase 3 – 505(b)(2) NDA	TBD
conbercept	Chengdu Kanghong	Wet AMD	Intraocular	Phase 3 – BLA	TBD
cortrophin (purified gel)	ANI	MS	IV	Phase 3 – sNDA	TBD
dalcetrapib	Dalcor	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 – NDA	TBD
daprodustat	GlaxoSmithKline	Anemia due to CKD (dialysis-dependent & dialysis-independent)	Oral	Phase 3 – NDA	TBD
dasiglucagon	Zealand	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 – NDA	TBD
dehydrated human amnion-chorion membrane	Mimedx	Achilles tendonitis; Plantar fasciitis	IV	Phase 3 – BLA	TBD
denosumab (biosimilar to Amgen's Prolia®)	Novartis	Osteoporosis/osteopenia	SC	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
dianhydrogalactitol	Delmar	Brain cancer	IV	Phase 3 – 505(b)(2) NDA; Fast Track; Orphan Drug	TBD
diazepam film	Aquestive	Seizure clusters	Oral Transmucosal	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
difelikefalin	Enteris	Pruritus (hemodialysis-related)	IV	Phase 3 – NDA; Breakthrough Therapy	TBD
digoxin immune Fab	AMAG	Eclampsia/pre-eclampsia prevention	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
dihydroergotamine	Satsuma	Migraine treatment	Intranasal	Phase 3 – 505(b)(2) NDA	TBD
dihydroergotamine mesylate	Impel Neuropharma	Migraine treatment	Intranasal	Phase 3 – 505(b)(2) NDA	TBD
d-methylphenidate prodrug	Kempharm	ADHD	Oral	Phase 3 – NDA	TBD
donaperminogene seltoplasmid	Helixmith	Diabetic peripheral neuropathy; Diabetic foot ulcers; Peripheral arterial disease	IM	Phase 3 – BLA; RMAT	TBD
dupilumab (Dupixent®)	Sanofi	Atopic dermatitis (ages 6–2 years); COPD; Esophagitis	SC	Phase 3 – sBLA; Breakthrough Therapy	TBD
durvalumab (Imfinzi®)	AstraZeneca	Bladder cancer (with tremelimumab); NSCLC (1st line, with tremelimumab); SCLC	IV	Phase 3 – sBLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 – NDA; Fast Track	TBD
dust mite immunotherapy	Stallergenes	Allergic rhinitis	SL	Phase 3 – BLA	TBD
edasalonexent	Catabasis	Duchenne muscular dystrophy	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
efgartigimod	Argenx	Myasthenia gravis	IV, SC	Phase 3 – BLA; Orphan Drug	TBD
eflapegrastim	Spectrum	Neutropenia/leukopenia	SC	Phase 3 – BLA	TBD
efpeglenatide	Hanmi	T2DM	SC	Phase 3 – NDA	TBD
elafibranor	Genfit	NASH	Oral	Phase 3 – NDA; Fast Track	TBD
elivaldogene tavalentivec (Lenti-D)	Bluebird bio	Adrenomyeloneuropathy (adrenoleukodystrophy)	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
empagliflozin (Jardiance®)	Boehringer Ingelheim	Diabetic nephropathy	Oral	Phase 3 – sNDA	TBD
entinostat	Syndax	Breast cancer	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD
EP-2101 vaccine	OSE Immunotherapeutics	NSCLC	SC	Phase 3 – BLA; Orphan Drug	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 – NDA	TBD
estetrol/drospirenone	Mayne	Contraception	Oral	Phase 3 – NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
etanercept (biosimilar to Amgen's Enbrel)	Coherus	RA; PSO	SC	Phase 3 – BLA	TBD
etrasimod	Arena	UC	Oral	Phase 3 – NDA	TBD
etrolizumab	Genentech	UC	IV, SC	Phase 3 – BLA; Orphan Drug	TBD
evinacumab	Regeneron	Dyslipidemia/ hypercholesterolemia	SC	Phase 3 – BLA; Breakthrough Therapy	TBD
fenfluramine (low dose)	Zogenix	Lennox-Gastaut syndrome	Oral	Phase 3 – NDA; Orphan Drug	TBD
fevipiprant	Novartis	Asthma	Oral	Phase 3 – NDA	TBD
fexapotide triflutate	Nymox	Benign prostatic hyperplasia	Intratumoral	Phase 3 – NDA	TBD
fezolinetant	Astellas	Menopause vasomotor symptoms	Oral	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
fluocinolone (Iluvien®)	Alimera	Uveitis (chronic non-infectious uveitis affecting the posterior segment of the eye)	Intraocular	Phase 3 – sNDA; Orphan Drug	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
fostemsavir	Viiv	HIV-1 infection	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track	TBD
fusidic acid	Arrevus	ABSSSI	Oral	Phase 3 – NDA; Orphan Drug; QIDP	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 – NDA	TBD
glatiramer acetate	Mylan	MS	IM	Phase 3 – NDA	TBD
GLPG1690	Galapos	Idiopathic pulmonary fibrosis	Oral	Phase 3 – NDA; Orphan Drug	TBD
glycopyrrolate MDI	AstraZeneca	Asthma; COPD	Inhaled	Phase 3 – NDA	TBD
glycopyrronium bromide (Seebri® Neohaler®)	Sumitomo Dainippon	Asthma	Inhaled	Phase 3 – sNDA	TBD
GS010	Gensight	Leber's hereditary optic neuropathy	Intraocular	Phase 3 – BLA; Orphan Drug	TBD
hydrocortisone (granules)	Diurnal	Congenital adrenal hyperplasia (pediatrics)	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
hydrogen peroxide (Eskata®)	Aclaris	Warts	Topical	Phase 3 – sNDA	TBD
ibrexafungerp	Scynexis	Fungal infections (systemic and non-systemic)	IV, Oral	Phase 3 – NDA; Fast Track; Orphan Drug; QIDP	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
iclaprim	Motif Bio	ABSSSI; HAP	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 – BLA; Breakthrough Therapy; Orphan Drug	TBD
immunoglobulin IV 10%	Prometic Life Sciences	Primary immunodeficiencies	IV	Phase 3 – BLA	TBD
inclisiran	The Medicines Company	Dyslipidemia/ hypercholesterolemia	SC	Phase 3 – NDA; Orphan Drug	TBD
infliximab (biosimilar to Janssen's Remicade)	Nichi-Iko	RA; AS; PSO; PsA; CD; UC	IV	Phase 3 – BLA	TBD
insulin aspart (follow-on to Novo Nordisk's Novolog)	Mylan	T1DM; T2DM	SC	Phase 3 – 505(b)(2) NDA	TBD
insulin aspart (follow-on to Novo Nordisk's Novolog®)	Sanofi	T1DM; T2DM	SC	Phase 3 – 505(b)(2) NDA	TBD
insulin glargine (follow-on to Sanofi's Lantus)	Gan & Lee	T1DM; T2DM	SC	Phase 3 – 505(b)(2) NDA	TBD
iodine I-131 monoclonal antibody	Actinium	Myeloablation prior to allogeneic HSCT to treat AML	IV	Phase 3 – BLA; Orphan Drug	TBD
ipatasertib	Genentech	Breast cancer; Prostate cancer	Oral	Phase 3 – NDA	TBD
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis (non-radiographic); PSO (pediatric)	SC	Phase 3 – sBLA	TBD
lacosamide (Vimpat®)	UCB	Partial seizures	IV, Oral	Phase 3 – sNDA	TBD
L-citrulline	Asklepion	Acute lung injury	IV	Phase 3 – NDA; Orphan Drug	TBD
lebrikizumab	Dermira	Atopic dermatitis	SC	Phase 3 – BLA	TBD
lentiviral beta-globin gene transfer (Zynteglo)	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
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
ligelizumab	Novartis	Urticaria	SC	Phase 3 – BLA	TBD
linaclotide (Linzess®)	Ironwood	IBS (treatment of abdominal symptoms)	Oral	Phase 3 – sNDA	TBD
linzagolix	Obseva	Endometriosis	Oral	Phase 3 – NDA	TBD
lisocabtagene maraleucel	Celgene	DLBCL	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug; RMAT	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
L-lactic acid/citric acid/ potassium bitartrate	Evofem	Contraception; UTI	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	Novartis	Prostate cancer	IV	Phase 3 – NDA	TBD
margetuximab	Macrogenics	Breast cancer	IV	Phase 3 – BLA; Fast Track	TBD
maribavir	Takeda	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
meloxicam/rizatriptan	Axsome	Migraine treatment	Oral	Phase 3 – 505(b)(2) NDA	TBD
melphalan prodrug	Oncopeptides	Multiple myeloma	IV	Phase 3 – NDA; Orphan Drug	TBD
meropenem/vaborbactam (Vabomere®)	Melinta	HAP; Septicemia	IV	Phase 3 – sNDA; QIDP	TBD
metachromatic leukodystrophy gene therapy	Orchard	Metachromatic leukodystrophy	IV	Phase 3 – BLA; Orphan Drug	TBD
microbiota suspension	Ferring	Recurrent *C. difficile* infection	Rectal	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
minocycline	Citius	Catheter-related bacteremia	IV	Phase 3 – 505(b)(2) NDA; Fast Track; QIDP	TBD
mirikizumab	Eli Lilly	PSO; UC	IV, SC	Phase 3 – BLA	TBD
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
nadofaragene firadenovec	Trizell	Mesothelioma	Percutaneous catheter injection	Phase 3 – BLA	TBD
nalbuphine ER	Trevi	Pruritus	Oral	Phase 3 – NDA	TBD
napabucasin	Sumitomo Dainippon	CRC	Oral	Phase 3 – NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
natalizumab (biosimilar to Biogen's Tysabri®)	Novartis	MS	IV	Phase 3 – BLA	TBD
nifurtimox	Bayer	Chagas disease	Oral	Phase 3 – NDA; Orphan Drug	TBD
nirsevimab	AstraZeneca	Respiratory syncytial virus prevention	Not specified	Phase 3 – BLA; Breakthrough Therapy; Fast Track	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 – NDA	TBD
nolasiban	Obseva	Female infertility	Oral	Phase 3 – NDA	TBD
octreotide acetate	Chiasma	Acromegaly	Oral	Phase 3 – 505(b)(2) NDA; Orphan Drug	TBD
olaparib (Lynparza®)	AstraZeneca	Breast cancer (metastatic, adjuvant treatment); Ovarian cancer (*gBRCAm* PSR); Ovarian cancer (recurrent PSR); Ovarian cancer (1st-line)	Oral	Phase 3 – sNDA	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
onasemnogene abeparvovac-xioi (Zolgensma®)	Novartis	Spinal muscular atrophy (Type 2/3)	IV, Intrathecal	Phase 3 – sBLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
ondansetron ER (once-daily)	Redhill	Gastroenteritis	Oral	Phase 3 – 505(b)(2) NDA	TBD
oportuzumab monatox	Sesen	Bladder cancer	Intravesical	Phase 3 – BLA; Fast Track	TBD
oxycodone ER	Zyla Life Sciences	Moderate to severe pain; Chronic low back pain	Intranasal, Oral	Phase 3 – 505(b)(2) NDA; Fast Track	TBD
ozanimod	Celgene	CD; UC	Oral	Phase 3 – NDA	TBD
paliperidone (6-month injectable)	Janssen	Schizophrenia	IM	Phase 3 – sNDA	TBD
palovarotene	Ipsen	Fibrodysplasia ossificans progressiva	Oral, Topical	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
pamrevlumab	Fibrogen	Idiopathic pulmonary fibrosis	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
pegunigalsidase alfa	Chiesi	Fabry disease	IV	Phase 3 – BLA; Fast Track	TBD
pemigatinib	Incyte	Biliary tract cancer (FGFR2 translocated, 2nd-line)	Oral	Phase 3 – NDA; Orphan Drug	TBD
pertuzumab (Perjeta®)	Genentech	Breast cancer (HER2+, adjuvant, with ado-trastuzumab)	IV	Phase 3 – sBLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
PF-06651600	Pfizer	Alopecia areata	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD
pimecrolimus	Bausch Health	Atopic dermatitis	Topical	Phase 3 – NDA	TBD
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)	Liminal	Hypoplasminogenemia	IV	Submitted – BLA; Fast Track; Orphan Drug; Rare Pediatric Disease	TBD
plinabulin	Beyondspring	NSCLC; Neutropenia/ leukopenia	IV	Phase 3 – NDA	TBD
pneumococcal 15-valent conjugate vaccine	Merck	Invasive pneumococcal disease prevention	IM	Phase 3 – BLA; Breakthrough Therapy	TBD
polatuzumab vedotin-piiq (Polivy®)	Genentech	DLBLC (1st-line)	IV	Phase 3 – sBLA; Breakthrough Therapy; Orphan Drug	TBD
pollinex quattro grass	Allergy Therapeutics	Allergic rhinitis	SC	Phase 3 – BLA	TBD
ponesimod	Janssen	MS	Oral	Phase 3 – NDA	TBD
ranibizumab (biosimilar to Genentech's Lucentis)	Samsung Bioepis	Wet AMD	Intraocular	Phase 3 – BLA	TBD
ranibizumab (biosimilar to Genentech's Lucentis)	Santo	Wet AMD	Intraocular	Phase 3 – BLA	TBD
ranibizumab (biosimilar to Genentech's Lucentis®)	STADA Arzneimittel	Wet AMD	Intraocular	Phase 3 – BLA	TBD
ravulizumab-cwvz (at-home, weekly SC formulation)(Ultomiris®)	Alexion	Paroxysmal nocturnal hemoglobinuria	IV, SC	Phase 3 – sBLA; Orphan Drug	TBD
relebactam/imipenem/ cilastatin	Merck	HAP	IV	Phase 3 – NDA; Fast Track; QIDP	TBD
relugolix	Myovant	Endometriosis; Uterine fibroids; Prostate cancer	Oral	Phase 3 – NDA	TBD
remestemcel-L	Mesoblast	GVHD (steroid-refractory, pediatric)	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 syncytial virus (RSV) nanoparticle vaccine	Novavax	RSV prevention	IM	Phase 3 – BLA; Fast Track	TBD
RG6206 (anti-myostatin adnectin)	Roche	Duchenne muscular dystrophy	SC	Phase 3 – BLA; Orphan Drug	TBD
ridinilazole	Summit	*C. difficile*-associated diarrhea/infection	Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
ripretinib	Deciphera	Gastrointestinal stromal tumor	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
risankizumab-rzaa (Skyrizi®)	Abbvie	CD; UC; PsA	SC	Phase 3 – sBLA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
risdiplam	Roche	Spinal muscular atrophy	Oral	Phase 3 – NDA; Breakthrough Therapy; 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	Phase 3 – BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Teva	RA; CLL/ SLL; NHL (indolent); ANCA-associated vasculitis	IV	Phase 3 – BLA	TBD
rivoceranib	LSK BioPartners	Gastric cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
ropeginterferon alfa-2b	Essentia	Polycythemia vera	SC	Phase 3 – BLA; Orphan Drug	TBD
roxadustat	AstraZeneca	Anemia due to CKD (dialysis-independent & dialysis-dependent); Anemia due to oncology treatment	Oral	Phase 3 – NDA	TBD
ruxolitinib	Incyte	Vitiligo	Topical	Phase 3 – NDA	TBD
ruxolitinib (Jakafi®)	Incyte	GVHD	Oral	Phase 3 – sNDA; Breakthrough Therapy; Orphan Drug	TBD
sacubitril/valsartan (Entresto®)	Novartis	Heart failure (preserved ejection fraction); Post acute myocardial infarction	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
secukinumab (Cosentyx®)	Novartis	Axial spondyloarthritis (non-radiographic)	IV, SC	Phase 3 – sBLA	TBD
seladelpar	Cymabay	Primary biliary cholangitis/hepatic fibrosis	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
selinexor (Xpovio™)	Karyopharm	Multiple myeloma (with bortezomib and dexamethasone); Sarcoma; Uterine cancer	Oral	Phase 3 – sNDA; Fast Track; Orphan Drug	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	SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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
sodium thiosulfate	Fennec	Chemotherapy-induced hearing loss	IV	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
sofpironium bromide	Brickell	Axillary hyperhidrosis	Topical	Phase 3 – NDA	TBD
somatrogon	Opko	Growth hormone deficiency	SC	Phase 3 – BLA; Orphan Drug	TBD
sotagliflozin	Lexicon	T2DM	Oral	Phase 3 – NDA	TBD
sparsentan	Retrophin	Focal segmental glomerulosclerosis	Oral	Phase 3 – NDA; Orphan Drug	TBD
sulopenem etzadroxil	Iterum	Uncomplicated UTI	IV, Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
sutimlimab	Sanofi	Cold agglutnin disease	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
suvodirsen	WAVE Life Sciences	Duchenne muscular dystrophy	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
tabelecleucel	Atara	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
tafasitamab	Morphosys	DLBCL	IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
tanezumab	Pfizer	Osteoarthritis; Chronic low back pain, cancer pain	IV, SC	Phase 3 – BLA; Fast Track	TBD
tapinarof	Roivant	PSO	Topical	Phase 3 – NDA	TBD
tasimelteon (Hetlioz®)	Vanda	Smith-Magenis syndrome	Oral	Phase 3 – sNDA; Orphan Drug	TBD
tecarfarin	Espero	Anticoagulation	Oral	Phase 3 – NDA	TBD
tecovirimat (Tpoxx®)	SIGA	Smallpox	IV	Phase 3 – sNDA; Fast Track; Orphan Drug	TBD
tenapanor (Ibsrela®)	Ardelyx	Hyperphosphatemia (in CKD patients on dialysis)	Oral	Phase 3 – sNDA	TBD
teprasiran	Quark	Delayed graft function; Kidney injury prevention following cardiac surgery	IV	Phase 3 – NDA	TBD
terlipressin	Mallinckrodt	Hepatorenal syndrome	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
tezepelumab	AstraZeneca	Asthma	SC	Phase 3 – BLA; Breakthrough Therapy	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
timbetasin	Regenerx	Dry eye syndrome	Topical	Phase 3 – NDA	TBD
tirbanibulin	Almirall	Actinic keratoses	Topical	Phase 3 – NDA	TBD
tirzepatide	Eli Lilly	T1DM	SC	Phase 3 – NDA	TBD
tisagenlecleucel-t (Kymriah™)	Novartis	CLL/SLL; DLBCL (1st relapse)	IV	Phase 3 – sBLA; Breakthrough Therapy; Orphan Drug	TBD
tonogenchoncel-L	Kolon Tissuegene	Osteoarthritis (knee)	Intraarticular	Phase 3 – BLA	TBD
tradipitant	Vanda	Gastroparesis	Oral	Phase 3 – NDA	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/pertuzumab	Genentech	Breast cancer	SC	Phase 3 – BLA	TBD
treprostinil	Liquidia	PAH	Inhaled	Phase 3 – 505(b)(2) NDA	TBD
tripotassium citrate monohydrate/potassium hydrogen carbonate micro-tablet	Advicenne	Renal tubular acidosis	Oral	Phase 3 – NDA	TBD
trivalent hepatitis B vaccine	VBI Vaccines	Hepatitis B virus (HBV) prevention	IM	Phase 3 – BLA	TBD
ublituximab	TG	CLL/SLL; MS	IV	Phase 3 – BLA; Orphan Drug	TBD
udenafil	Allergan	Congenital single ventricle heart disease (adolescents)	Oral	Phase 3 – NDA; Orphan Drug	TBD
umbralisib	TG	CLL/SLL; DLBCL; Indolent NHL; Marginal zone lymphoma	Oral	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
upadacitinib (Rinvoq™)	Abbvie	Atopic dermatitis; Axial spondyloarthritis; PsA; CD; UC	Oral	Phase 3 – sNDA; Breakthrough Therapy	TBD
vadadustat	Akebia	Anemia due to CKD (dialysis-dependent & dialysis-independent)	Oral	Phase 3 – NDA	TBD
valoctocogene roxaparvovec	Biomarin	Hemophilia A	Intratumoral, IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
vibegron	Urovant	Overactive bladder	Oral	Phase 3 – NDA	TBD
vilanterol trifenatate	GlaxoSmithKline	Asthma; COPD	Inhaled	Phase 3 – NDA	TBD
viloxazine	Supernus	ADHD	Oral	Phase 3 – NDA	TBD
visomitin	Mitotech	Dry eye syndrome	Ophthalmic	Phase 3 – NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
vocimagene amiretrorepvec	Tocen	Brain cancer (malignant glioma; glioblastoma)	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	Dyslipidemia/ hypercholesterolemia; Lipodystrophy	SC	Phase 3 – NDA; Orphan Drug	TBD
vosoritide	Biomarin	Achondroplasia	SC	Phase 3 – NDA; Orphan Drug	TBD
vutrisiran	Alnylam	Transthyretin amyloid cardiomyopathy (wild type or hereditary); Transthyretin amyloid polyneuropathy	SC	Phase 3 – NDA; Orphan Drug	TBD
zilucoplan	Ra	Myasthenia gravis	SC	Phase 3 – NDA; Orphan Drug	TBD
zoliflodacin	Entasis	Urinary tract and reproductive tract infections (antibacterial)	Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
zolmitriptan (micro-needle patch)	Zosano	Migraine treatment	Transdermal	Phase 3 – 505(b)(2) NDA	TBD
zuranolone (SAGE-217)	Sage	MDD; Insomnia	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track	TBD

Complete Response Letter (CRL)/Withdrawn Drugs

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
budesonide/formoterol fumarate/glycopyrronium	AstraZeneca	COPD	Inhaled	CRL	TBD
filgrastim (biosimilar to Amgen's Neupogen)	Tanvex	Neutropenia/leukopenia	SC	CRL	TBD
golodirsen	Sarepta	Duchenne muscular dystrophy	IV	CRL	TBD
insulin glargine (follow-on to Sanofi's Lantus)	Mylan/Biocon	T1DM; T2DM	SC	CRL	TBD
loteprednol etabonate 0.25%	Kala	Dry eye syndrome	Topical	CRL	TBD
rizatriptan film	Gensco	Migraine treatment	Oral transmucosal	CRL	TBD
tasimelteon (Heltlioz)	Vanda	Jet lag disorder	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

ART Antiretroviral Therapy

ARV Antiretroviral

AS Ankylosing Spondylitis

AST Aspartate Aminotransferase

BLA Biologics License Application

BsUFA Biosimilar User Fee Act

CABP Community Acquired Bacterial Pneumonia

CAP Community Acquired Pneumonia

CD Crohn's Disease

CDC Centers for Disease Control and Prevention

CF Cystic Fibrosis

CHF Congestive Heart Failure

CI Confidence Interval

CKD Chronic Kidney Disease

CLL Chronic Lymphocytic Leukemia

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

DOR Duration of Response

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

HAM-D Hamilton Depression Rating Scale

HAP Healthcare-Associated Pneumonia

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

HR Hazard Ratio

HSCT Hematopoietic Stem Cell Transplant

HTN Hypertension

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

MRI Magnetic Resonance Imaging

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

ORR Overall/Objective Response Rate

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

RNA Ribonucleic Acid

RTOR Real-Time Oncology Review

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

SL Sublingual

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

SNRI Serotonin and Norepinephrine Reuptake Inhibitor

SSRI Selective Serotonin 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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