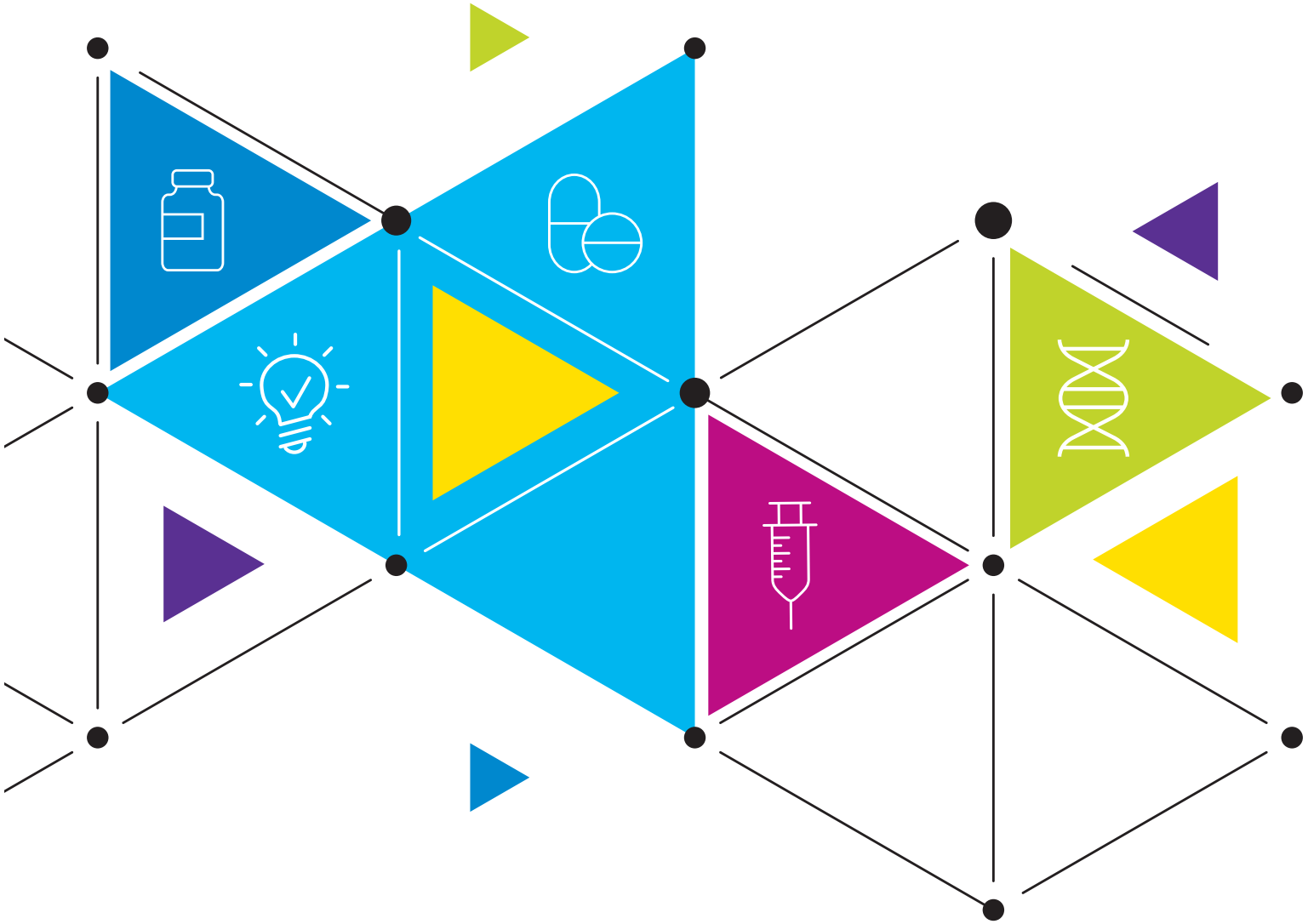
January 2020

MRx PIPELINE

A VIEW INTO UPCOMING SPECIALTY & TRADITIONAL DRUGS



Magellan Rx MANAGEMENT℠

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INTRODUCTION

Welcome to the *MRx Pipeline.* In its fourth 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 2024. 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.

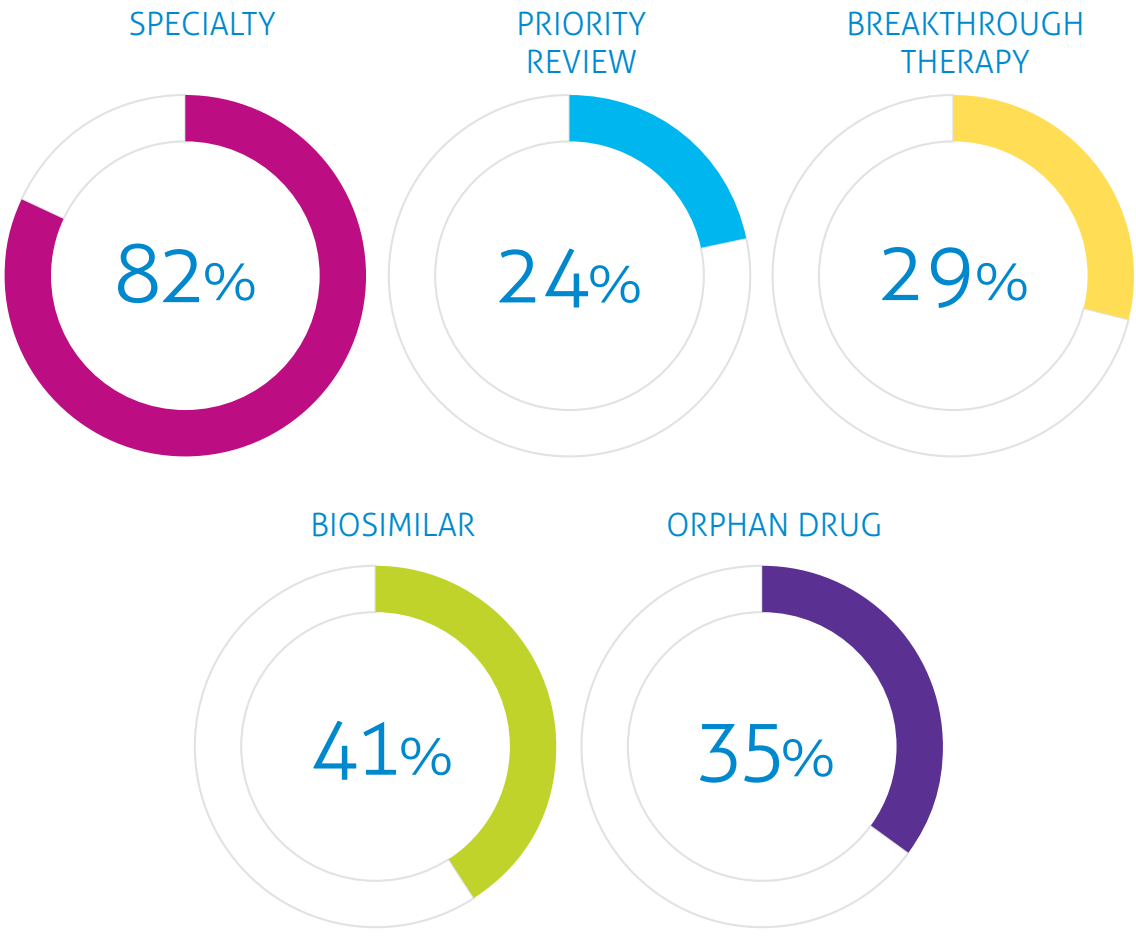
In the past few years, game changers, such as products in the hepatitis C field and chimeric antigen receptor (CAR)-T therapies, have revolutionized standard of care. In 2019, the FDA approved 48 novel drugs including the first treatment for postpartum depression, the record-breaking 1-time infusion gene therapy for select pediatric patients with spinal muscular atrophy, and 10 new biosimilars. The agency utilized Project Orbis, an ongoing collaboration with its international partners to provide a framework for simultaneous submission and review of oncology drug applications. Last year, the first NDA was approved under the real-time oncology review (RTOR) pilot. As part of this program, the FDA reviews clinical data prior to the formal submission of the drug application, leading to an approval well in advance of the FDA goal date.

As we look ahead, a continued trend toward the approval of specialty medications, drugs for rare diseases, growth of biosimilars, digital therapeutics, and new treatment modalities using gene therapy are expected. Noteworthy pipeline trends to watch in 2020 include the first drugs for peanut allergies, first gene therapy for hemophilia A, first treatment for NASH, and the transition of insulins and growth hormones from drugs to biologics. In the upcoming quarters, development of complex therapies, therapeutic options for rare hereditary diseases, oncology, immunology, neurology, cardiology, and investigational agents will be monitored on the MRx radar. Moreover, sprouting products for ophthalmology, hematology, and women's health 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.

abicipar pegol *intraocular*

Allergan

PROPOSED INDICATIONS

Neovascular (wet) age-related macular degeneration (AMD)

CLINICAL OVERVIEW

Abicipar pegol is an ankyrin repeat protein (DARPin)-based VEGF inhibitor designed to decrease angiogenesis, vascular permeability, and inflammation in the eye.

Two identical, phase 3, double-blind trials (CEDAR [n=939] and SEQUOIA [n=949]) compared the safety and efficacy of abicipar pegol and ranibizumab in treatment-naïve patients with wet AMD. At week 52, 8- and 12-week regimens of abicipar pegol were non-inferior to monthly doses of ranibizumab, as measured by the proportion of patients with stable vision (CEDAR, 91.7%, 91.2%, and 95.5%, respectively; SEQUOIA, 94.8%, 91.3%, and 96%, respectively). Stable vision was defined as < 15-letter loss in BCVA. Across the respective treatment regimens, mean gain in BCVA from baseline was 6.7, 5.6, and 8.5 letters in CEDAR and 8.3, 7.3, and 8.3 letters in SEQUOIA; the mean change in central retinal thickness (CRT) was -142 µm, -150 µm, and -141 µm in CEDAR and -147 µm, -142 µm, and -147 µm in SEQUOIA. Pooled data at 104 weeks demonstrated a durable response. At week 52, overall, intraocular inflammation was reported in 15.4%, 15.3%, and 0.3% of patients in each study arm, respectively; however, the incidence was similar between the groups at week 104 (0.8%, 2.3%, and 1%, respectively).

In both studies, abicipar pegol 2 mg was administered via intravitreal injection into the study eye on day 1, week 4, and week 8, followed by every 8 weeks thereafter (8-week regimen), or on day 1, week 4, and week 12, followed by every 12 weeks thereafter (12-week regimen). Ranibizumab 0.5 mg was administered intravitreally every 4 weeks. In all treatment arms, patients received therapy through week 96.

PLACE IN THERAPY

AMD is a leading cause of irreversible 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 from dry (non-neovascular) AMD to wet AMD when new blood vessels form behind the retina. The new vessels are fragile, leaking both fluid and blood which leads to scarring and loss of vision.

Intravitreal administration of VEGF inhibitors is considered first-line treatment for wet AMD. Several VEGF inhibitors are available, including aflibercept (Eylea®), pegaptanib (Macugen®), ranibizumab (Lucentis®), and brolucizumab (Beovu®), as well as off-label use of the less costly bevacizumab (Avastin®). Biosimilars to Avastin (Mvasi™, Zirabev™) are also available in the US. Approved VEGF inhibitors are administered by an HCP every 4 weeks (aflibercept, ranibizumab), every 6 weeks (pegaptanib), or every 8 to 12 weeks (brolucizumab). Clinical trials showed similar efficacy of abicipar pegol to ranibizumab with significantly fewer maintenance injections (4 versus 12 per year). During the first year of therapy, abicipar pegol was associated with greater intraocular inflammation compared to ranibizumab, but this difference diminished in the second year.

Potential advantages of abicipar pegol's DARPin platform over traditional monoclonal antibody VEGF inhibitors include high VEGF affinity, low immunogenicity, and easier manufacturing. If approved, abicipar pegol's lower injection burden and potential lower cost may allow it to compete in the crowded AMD market space. Additionally, several VEGF inhibitors are in late-stage development, including an ophthalmic formulation of bevacizumab, biosimilars to ranibizumab intravitreal injection, and an intravitreal implanted formulation of ranibizumab.

FDA APPROVAL TIMELINE

June–July 2020

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$6	$15	$25	$36	$47

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

exenatide implantable mini-pump (ITCA-650) *SC*

Intarcia/Janssen

PROPOSED INDICATIONS

Type 2 diabetes mellitus (T2DM)

CLINICAL OVERVIEW

Exenatide drug/device is a subdermally implanted osmotic mini-pump that uses the Medici System™ to provide continuous SC administration of the GLP-1RA exenatide immediate-release.

Safety and efficacy of exenatide continuous administration (ITCA-650) were established in the phase 3 FREEDOM clinical trial program in patients with T2DM who were not controlled on their baseline antidiabetic regimen (HbA1c, 7% to 10%). In all trials, the initial dose of exenatide was 20 mcg/day for 13 weeks, which was then titrated upward based on the study protocol. In the FREEDOM-1 trial (n=460), at week 39, exenatide at continuous doses of 40 and 60 mcg/day demonstrated significantly greater mean reductions in HbA1c from baseline compared to placebo (-1.1% and -1.2%, respectively; p=0.001 for both doses). In FREEDOM-2 (n=535), exenatide 60 mcg/day was statistically superior to oral sitagliptin 100 mg daily in HbA1c reduction (-1.5% versus -0.8%, respectively; p<0.001) and weight loss (-4 kg versus -1.3 kg, respectively; p<0.001) over 52 weeks. A significantly greater proportion of patients achieved HbA1c < 7% with exenatide versus sitagliptin (61% versus 42%, respectively; p<0.001) and fewer patients on exenatide required rescue therapy (15% versus 35%, respectively). The FREEDOM-CVO placebo-controlled safety trial (n>4,000) assessed CV risk of exenatide 60 mcg/day in patients with a history of coronary, cerebrovascular, or peripheral artery disease or multiple CV risk factors. The mean treatment duration was 1.2 years. FREEDOM-CVO met its primary endpoint and demonstrated non-inferiority for CV safety. While the open-label FREEDOM-1 HBL study in patients (n=60) with a high baseline HbA1c (10% to 12%) demonstrated efficacy of exenatide continuous administration in HbA1c and weight reduction, the study was stopped early due to concerns of long-term stability and sterility of the product; no root cause has been communicated. In the FREEDOM-3S trial, patients retained glycemic control and experienced significant weight loss when switched from liraglutide 1.2 mg or 1.8 mg directly to the full continuous exenatide dose. The overall safety of continuous exenatide is consistent with other injectable GLP-1RAs.

PLACE IN THERAPY

Exenatide implantable mini-pump (ITCA-650) is a single-use matchstick-sized device (4 mm x 45 mm) that is inserted subdermally into the abdomen by an HCP. Safety and efficacy of continuous delivery of exenatide provided by the device is consistent with other GLP-1RAs. The estimated adherence to daily or weekly SC administered GLP-1RA ranges from 38% to 54%. Recently approved semaglutide (Rybelsus®) provides the only GLP-1RA oral formulation in an otherwise injectable class. This may improve adherence, but timing of the Rybelsus dose (≥ 30 minutes prior to first food, beverage, or other medication of the day) should be considered. The exenatide implantable mini-pump, designed to be replaced every 6 to 12 months, may also facilitate compliance since it provides a convenient continuous SC dose of exenatide without the need for daily or weekly injections or concern of interaction of co-administered food or drug. Exenatide levels rapidly decline within 24 hours of removing the device by an HCP; therefore, therapy may be promptly discontinued, if needed.

If approved, exenatide continuous administration could compete as a second- and third-line therapy for T2DM. Intarcia is also developing the Medici System for HIV prophylaxis (once or twice per year drug delivery) as well as for the management of autoimmune and inflammatory diseases.

FDA APPROVAL TIMELINE

March 9, 2020

FINANCIAL FORECAST

The projected annual US sales for exenatide mini-pump (ITCA-650) are currently not available.

fenfluramine low-dose *oral*

Zogenix

PROPOSED INDICATIONS

Dravet syndrome

CLINICAL OVERVIEW

Fenfluramine stimulates the release and inhibits the reuptake of serotonin in the brain.

A double-blind, placebo-controlled trial (Study 1501) evaluated 2 doses of fenfluramine oral liquid added to current AED therapy in 119 patients 2 to 18 years of age with Dravet syndrome. The mean monthly convulsant seizure frequency (MCSF) at baseline was approximately 40 seizures. After 14 weeks, the study reported greater reductions compared to placebo in mean MCSF by 62.3% (95% CI, -47.7 to -72.8; p<0.0001) with fenfluramine 0.7 mg/kg/day (maximum daily dose of 26 mg) and 32.4% (95% CI, -6.2 to -51.3; p=0.0209) with fenfluramine 0.2 mg/kg/day. More patients achieved ≥ 50% reduction in seizure frequency with fenfluramine 0.7 mg/kg/day and 0.2 mg/kg/day compared to placebo (68%, 38%, and 12%, respectively). The median longest seizure-free time span achieved in each group was 25, 15, and 9.5 days, respectively. There were no signs of PAH or cardiac valve dysfunction. The most common adverse effects with fenfluramine, reported in ≥ 10% of patients, were decreased appetite, diarrhea, fatigue, lethargy, somnolence, and decreased weight. Another trial (Study 1504) evaluated fenfluramine in patients (n=87; 2 to 19 years of age) on background stiripentol. After 12 weeks at a stable dose, fenfluramine 0.4 mg/kg/day (maximum 17 mg/day) led to a 54% greater reduction in mean MCSF compared to placebo (95% CI, 35.6% to 67.2%; p<0.001).

PLACE IN THERAPY

Dravet syndrome is a rare, catastrophic infantile-onset epilepsy characterized by frequent, disabling seizures. It is reported in approximately 1 out of 15,700 individuals in the US. The average age of death is around 8 years of age (range, infancy to 18 years). The condition is very difficult to treat with existing AEDs. If approved, this low-dose of fenfluramine will be the third medication approved in the US for the treatment of Dravet syndrome in combination with other AEDs, following the approval of oral cannabidiol (Epidiolex®) and oral stiripentol (Diacomit®). Both cannabidiol and stiripentol, the latter of which targets gamma-aminobutyric acid $(GABA)_A$ and is for use only in patients also taking clobazam, are given twice daily for the treatment of Dravet and Lennox-Gastaut syndromes in patients ≥ 2 years of age. Both agents are associated with somnolence and risk of suicide. Stiripentol also carries a risk of neutropenia/thrombocytopenia and decreased appetite/weight, and cannabidiol is associated with hepatocellular injury. While the cannabidiol formulation lacks the psychoactivity of tetrahydrocannabinol (THC), it is classified as a schedule V controlled substance.

Historically, cardiac effects have been a significant concern with fenfluramine when used as a weight-loss agent (it was sold on its own and in combination with phentermine ["Fen-Phen"] but withdrawn from the market in 1997). No safety signals related to cardiotoxicities have been detected to date in the current, low-dose studies of fenfluramine for Dravet syndrome; however, its long-term safety remains to be elucidated. Additionally, the effect on appetite and weight may be problematic in some underweight patients with Dravet syndrome. In clinical studies, fenfluramine oral liquid demonstrated dose-dependent decreases in seizure frequency in patients with Dravet syndrome. Fenfluramine is also in phase 3 clinical trials for Lennox-Gastaut syndrome; top-line data are expected in the first quarter of 2020.

FDA APPROVAL TIMELINE

March 25, 2020

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$60	$96	$137	$174	$219

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

BACKGROUND

Neuromyelitis optica spectrum disorder (NMOSD), also known as Devic's syndrome, is a chronic autoimmune disorder characterized by demyelination and inflammation of the brain stem, spinal cord (myelitis), and optic nerve (optic neuritis). Pathophysiology of NMOSD includes defects in B lymphocyte (B cell)-mediated suppression of autoimmune responses; therefore, depletion of B cells by targeting CD19, a B cell surface marker, is a promising mechanism of therapy for NMOSD. In addition, approximately two-thirds of cases are associated with autoantibodies to anti-qauaporin-4 (AQP4-IgG) causing astrocyte damage in the brain and spinal cord. Notably, autoantibody-mediated tissue damage often leads to the release of inflammatory cytokines, such as interleukin (IL)-6, which is thought to play a key role in triggering inflammation in NMOSD and can serve as an additional target to treat NMOSD.

Patients with NMOSD may experience ophthalmic or neurologic symptoms at onset. Ophthalmic symptoms include unilateral or bilateral eye pain and loss of visual acuity. Neurologic symptoms include pain in the spine or limbs, mild to severe paralysis of the lower limbs, and loss of bowel and bladder control. Early stages of NMOSD may be confused with MS. Most patients experience repeated symptomatic episodes separated by periods of remission.

inebilizumab *IV*

Viela Bio

PROPOSED INDICATIONS

Neuromyelitis optica (Devic's syndrome)

CLINICAL OVERVIEW

Inebilizumab is a monoclonal antibody that targets CD19 on B cells leading to B cell depletion.

The phase 2/3, double-blind N-MOmentum trial randomized (3:1) 230 adults with NMOSD to inebilizumab or placebo monotherapy. The majority of patients were female (91%) and AQP4-IgG-seropositive (91%). The primary endpoint was an NMOSD attack, defined as new or worsening NMOSD symptoms per protocol-specified criteria. At 6.5 months, inebilizumab demonstrated a 72.8% relative risk reduction in the rate of NMOSD attack compared to placebo (attack rate, 12% versus 39%, respectively); relative risk reduction in AQP4-IgG-seropositive patients was 77.3%. At 1 year, 85% of patients treated with inebilizumab were episode-free. Inebilizumab also significantly reduced the risk of worsening disability, as measured by the EDSS and modified Rankin scale, number of total active MRI lesions (44% reduction; rate ratio 0.566), and NMOSD-related hospitalizations. There was no difference in visual acuity between the treatment groups. Due to a clear evidence of efficacy, the independent data monitoring committee recommended to stop the randomized study period early; an open-label extension trial is ongoing. In the combined blinded and open-label periods (mean treatment duration, 1.5 year; range, 0.2 to 3.7 years), serious adverse events were reported in 12% of inebilizumab-treated patients. The most commonly reported adverse effect was UTI (2.2%). Two deaths were reported during the open-label period, one of which was due to NMOSD and the other to probable inflammatory brain lesions of unknown etiology.

Inebilizumab 300 mg was administered IV on days 1 and 15 of a 6.5-month treatment period. Treatment was continued every 6 months during the open-label extension period.

FDA APPROVAL TIMELINE

June–July 2020

✓ Breakthrough Therapy ✓ Orphan Drug

FINANCIAL FORECAST

The projected annual US sales for inebilizumab are currently not available.

satralizumab *sc*

Genentech

PROPOSED INDICATIONS

Neuromyelitis optica (Devic's syndrome)

CLINICAL OVERVIEW

Satralizumab is a humanized recycling anti-IL-6 receptor monoclonal antibody.

The phase 3, double-blind SAkuraSky trial randomized (1:1) 83 patients ages 13 to 73 years with NMOSD to satralizumab or placebo, both in addition to background immunosuppressant therapy. Patients enrolled could be seropositive or seronegative for AQP4-IgG. Relapse was defined as the presence of new or worsening objective neurologic symptoms that included protocol-specified increases in EDSS and functional-system scores. Relapse occurred in 43% of placebo-treated patients, regardless of serotype. In those treated with satralizumab, relapse rate was 11% in AQP4-IgG-seropositive patients and 36% in AQP4-IgG-seronegative patients (20% overall). Duration of response was reported at 144 weeks. There were no differences between satralizumab or placebo regarding effect on pain or fatigue, as measured by the VAS pain score and Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-F) score, respectively. Incidence of serious adverse effects, including infection, were similar between treatment groups. Interim data of the SAkuraStar trial reported positive results with satralizumab monotherapy in adults with NMOSD (relapse risk reduction: 55% overall; 74% in AQP4-IgG-seropositive patients).

Satralizumab was administered SC at a dosage of 120 mg at weeks 0 and 2 and every 4 weeks thereafter.

FDA APPROVAL TIMELINE

April–May 2020

✓ Breakthrough Therapy ✓ Orphan Drug

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$14	$44	$97	$128	$151

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

PLACE IN THERAPY

NMOSD affects approximately 15,000 people in the US and occurs predominately in females. Disease onset can occur at any age. Only 3% of patients with NMOSD have a family history of the condition; however, about 50% of cases have a strong association with a personal or family history of autoimmune disease.

Acute NMOSD attacks are typically treated with high-dose IV corticosteroids, and plasma exchange may be used in patients who do not respond to steroid therapy. In June 2019, the IV-administered complement inhibitor eculizumab (Soliris®) became the first agent approved in the US to treat NMOSD in AQP4-IgG-seropositive adults. After 5 weekly loading doses, maintenance therapy is given every 2 weeks. Prior to eculizumab availability, long-term use of immunosuppressive agents (e.g., azathioprine, mycophenolate mofetil), low-dose corticosteroids, or rituximab (which mediates B cell lysis) were the only options for disease management. While NMOSD may mimic MS, drugs to treat MS are not effective in NMOSD.

If approved, inebilizumab and satralizumab could compete with eculizumab for the maintenance treatment of NMOSD. Inebilizumab and satralizumab demonstated efficacy in AQP4-IgG seropositive and seronegative patients; however, eculizumab is indicated only in adults who are anti-AQP4 antibody positive. In non-comparative studies, the overall risk reduction for NMOSD attack was nearly 73% with inebilizumab, up to 62% with satralizumab, and 94% with eculizumab. While satralizumab may provide a more convenient self-administered monthly SC option in adult and adolescent patients, inebilizumab was studied as two IV doses in a 6-month period in adults only.

ENDOCRINE

obeticholic acid (Ocaliva®) *oral*

Intercept

PROPOSED INDICATIONS

Non-alcoholic steatohepatitis (NASH)

CLINICAL OVERVIEW

Obeticholic acid is a potent and selective farnesoid X receptor (FXR) agonist. Activation of FXR by obeticholic acid inhibits lipogenesis, leading to a decrease in lipid accumulation in the liver.

The ongoing phase 3, double-blind REGENERATE trial evaluated the safety and efficacy of obeticholic acid in adults (n=1,968) with NASH and fibrosis stage F1 (with ≥ 1 accompanying comordidity), F2, or F3. At 18 months, the proportion of patients who achieved fibrosis improvement (≥ 1 stage) with no worsening of NASH was 18% (p=0.045) and 23% (p=0.0002) with oral once-daily obeticholic acid 10 mg and 25 mg, respectively, and 12% with placebo. The endpoint of NASH resolution with no worsening of liver fibrosis was not met in any group. The most common adverse effect with obeticholic acid 10 mg and 25 mg was pruritus, including severe cases (overall incidence, 28% and 51%, respectively). As seen in other NASH studies, LDL-C levels peaked (+22.6 mg/dL) at 4 weeks of obeticholic acid therapy and subsequently declined, approaching baseline at month 18.

PLACE IN THERAPY

NASH is a type of nonalcoholic fatty liver disease (NAFLD). NASH involves not only accumulation of fat in the liver (steatosis) but also hepatic inflammation and fibrosis. It is a progressive disease closely associated with metabolic disorders, such as obesity, metabolic syndrome, and diabetes. The prevalence of NAFLD in the US is estimated at 30% of the adult population, and NASH accounts for approximately 5% of NAFLD cases (15 million). Moreover, incidence is rising in pediatrics, due in part to an increase in childhood obesity. NASH is estimated to become the leading cause of liver transplantation in the US as early as 2020. Among diabetic Americans, it is estimated that over 70% have NAFLD and over half may have NASH. NASH usually presents in the fifth and sixth decades of life when irreversible liver damage becomes evident.

There are no FDA-approved drugs to treat NASH. Current treatment consists of lifestyle management (weight loss, exercise, alcohol avoidance) as well as diabetes, hyperlipidemia, and hypertension management. In clinical trials, obeticholic acid 25 mg demonstrated an improvement in fibrosis (> 1 stage) but did not provide resolution of NASH. Improvements in inflammation and fibrosis, as well as increases in insulin sensitivity, were also reported in a small trial in patients (n=64) with NASH and T2DM.

Obeticholic acid is poised to become the first medication approved in the US to treat NASH. Modest efficacy results may limit its uptake to patients with stage F2 or F3 fibrosis. The increase in LDL-C levels seen with the agent is of particular concern since patients with NASH are at higher risk for CV disease; therefore, concurrent antihyperlipidemic therapy may be needed. Obeticholic acid, at doses of 5 mg to 10 mg daily, is currently indicated to treat primary biliary cholangitis (PBC) and carries a boxed warning of increased risk of liver injury and death with higher than recommended dosages in patients with PBC and hepatic impairment (Child-Pugh class B or C); however, an increased risk of liver injury has not been reported in clinical trials for NASH.

Obeticholic acid is also being studied in patients with NASH with compensated cirrhosis (F4). Although, if the late-stage pipeline drugs aramchol, cenicrivioc, elabibranor, and MGL-3196 prove to be more effective or better tolerated for NASH, the window of opportunity for obeticholic acid as a first-line NASH agent may be limited.

FDA APPROVAL TIMELINE

June 26, 2020

✓ Breakthrough Therapy ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$35	$90	$205	$431	$762

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

opicapone *oral*

Neurocrine Biosciences

PROPOSED INDICATIONS

Parkinson's disease (PD) "off" episodes

CLINICAL OVERVIEW

Opicapone is a selective catechol-O-methyltransferase (COMT) inhibitor.

Two phase 3, placebo-controlled trials, BIPARK-1 and BIPARK-2, evaluated the safety and efficacy of opicapone in patients with PD with motor fluctuations ("off" episodes). Pooled data from the 14- to 15-week, double-blind periods of both trials demonstrated that opicapone 50 mg added to levodopa resulted in a significant decrease from baseline in off-time compared to placebo (-2.22 ± 0.17 versus -1.28 ± 0.17 hours, respectively; $p<0.001$). The response of opicapone was sustained for up to 1 year in the open-label extension phases of both trials. The BIPARK-1 trial also included an entacapone study arm and demonstrated noninferiority of opicapone 50 mg to entacapone 200 mg (off-time reduction, 60.8 versus 40.3 minutes, respectively). Moreover, patients who switched from entacapone to opicapone in the extension study experienced a 0.65 hour decrease in mean off-time. The most commonly reported treatment-related adverse effect with opicapone 50 mg was dyskinesia (20.4%, pooled data), and approximately 3% of patients discontinued treatment due to this side effect. Hepatic injury and severe diarrhea were not observed with opicapone.

Opicapone was administered as once-daily oral dosages of 5 mg (BIPARK-1 only), 25 mg, and 50 mg. Unlike the 50 mg dose, the 5 mg and 25 mg dosages did not provide statistically significant responses.

PLACE IN THERAPY

It is estimated that 1 million people are diagnosed with PD in the US. Carbidopa/levodopa (CD/LD) is the mainstay of treatment; however, over time its effect diminishes, resulting in motor fluctuations ("off" episodes) in approximately 40% to 60% of PD patients. The "off" episodes affect the patient's ability to perform ADLs and often worsen with disease progression. CD/LD dose modification and medication supplementation (e.g., dopamine agonists, COMT inhibitors, or MAO-B inhibitors) may reduce off-time; however, these strategies may not be as helpful in advanced PD. New formulations of older drugs 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. In addition, the first-in-class, once-daily, oral adenosine A2a receptor antagonist istradefylline (Nourianz™) was approved as adjunct to CD/LD to treat motor fluctuation in patients with PD; however, its path to FDA approval was problematic due to mixed results in clinical trials.

COMT inhibitors increase delivery of levodopa to the brain by limiting its peripheral metabolism. Currently available COMT inhibitors indicated as adjunct to CD/LD to reduce "off" episodes include entacapone (Comtan®, Stalevo® [fixed-dose combination with CD/LD]) and tolcapone (Tasmar®). While tolcapone carries a boxed warning of the risk for potentially fatal hepatotoxicity, opicapone and entacapone are not associated with this effect to date. In clinical trials, opicapone 50 mg demonstrated noninferiority to entacapone and led to an improvement in off-time in patients who switched from entacapone. If approved, opicapone, a once-daily, third-generation COMT inhibitor, may provide a safe and effective option in the PD armamentaria. Sunovion's apomorphine sublingual film is also in the pipeline for "off" episodes, with an anticipated FDA action date of May 21, 2020.

FDA APPROVAL TIMELINE

April 24, 2020

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$13	$49	$94	$144	$180

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

risdiplam *oral*

Genentech

PROPOSED INDICATIONS

Spinal muscular atrophy (SMA) types 1, 2, and 3

CLINICAL OVERVIEW

SMA is a rare, debilitating disease characterized by progressive motor function decline and muscular atrophy while sparing cognitive abilities. It is caused by deletion or mutation of the survival of motor neuron (SMN)-1 gene (SMN1), resulting in a lack of SMN protein needed to maintain motor neurons in the spinal cord and lower brain stem. A second gene, SMN2, produces low levels of the SMN protein that are insufficient to fully compensate for the lack of SMN1. SMA is classified into phenotypes that are distinguished by age of onset, severity, and prognosis, which mainly depend on the number of SMN2 gene copies. Fewer copies of SMN2 result in more severe disease.

Risdiplam is a survival of motor neuron-2 (SMN2) splicing modifier designed to increase and sustain SMN protein levels in the CNS and peripheral tissues of the body. It is currently being studied to treat SMA types 1, 2, and 3. Risdiplam is an oral liquid that is administered once daily.

FDA submission of risdiplam is supported by 2 pivotal clinical trials. In both, patients were excluded if they had prior or concurrent treatment with an SMN-targeting antisense oligonucleotide, SMN2 splicing modifier therapy, gene therapy, or a history of cell therapy. The phase 2/3 FIREFISH single-arm study enrolled 21 patients 1 to 7 months of age with SMA type 1 who did not require tracheostomy or invasive or awake non-invasive ventilation. After 16 months of treatment, 86% of patients were event-free and did not need permanent ventilation. Among the milestone achievements, 48% were able to maintain upright head control and 33% could sit independently. While no treatment-related safety trends were identified, pneumonia was reported in 4 infants, and respiratory tract infection and acute respiratory failure/distress each were reported in 2 infants. Three deaths occurred, 1 of which occurred after risdiplam was discontinued.

Part 2 of the SUNFISH trial enrolled 180 patients 2 to 25 years of age with SME type 2 or 3. The study met its primary endpoint of change from baseline in the Motor Function Measure 32 scale after 1 year of treatment with risdiplam, compared to placebo. No new safety signals were reported.

PLACE IN THERAPY

SMA is the leading genetic cause of infant mortality. Currently, several types of SMA have been identified and vary by age of onset, severity, and prognosis. SMA type 1 is the most common phenotype, reported in about 1 in 10,000 newborns. Onset is soon after birth to 6 months of age. Patients with SMA type 1 typically do not achieve motor milestones and require nutritional support, with or without respiratory support, by age 12 months. Onset of SMA type 2 is usually between 3 to 15 months of age, and lower limb muscle weakness is common. Type 3 accounts for 30% of all SMA cases, with symptoms appearing at 18 months of age or later. Individuals with type 3 achieve independent ambulation that can diminish over time.

If approved, the small-molecule risdiplam will be the third disease-modifying therapy (DMT) available in the US to treat SMA. It will follow approval of the biologic agents nusinersen (Spinraza®; December 2016), a SMN2-directed antisense oligonucleotide indicated to treat all SMA types in patients from birth to adulthood, and onasemnogene abeparvovec-xioi (Zolgensma®; May 2019), a gene replacement therapy indicated for all types of SMA in patients < 2 years of age. Separate non-comparative studies of the 3 agents reported improvement in motor control in patients with SMA type 1, as assessed by the CHOP-INTEND score (scale 0-64, with higher numbers conferring higher function). The proportion of patients who achieved a CHOP-INTEND score ≥ 40 was 82% with risdiplam, 71% with nusinersen, and 95% with onasemnogene abeparvovec-xioi.

PLACE IN THERAPY continued

Risdiplam's oral, once-daily dosing will provide a significant advantage to this field. It is the first SMA agent to allow for self- or caregiver-administration. It also lacks the administration issues of intrathecal nusinersen, which requires maintenance doses every 4 months that must be given in an institutional setting. Also, while onasemnogene abeparvovec-xioi is designed to be a 1-time IV-infusion, durability of its effect remains to be seen. To date, risdiplam appears to be well tolerated. In contrast, safety concerns for nusinersen include increased risk for bleeding and renal toxicity, and onasemnogene abeparvovec-xioi carries a boxed warning for acute serious liver injury.

The FIREFISH and SUNFISH trials support the use of risdiplam for treatment-naïve, symptomatic patients ages 1 month to 25 years (depending on phenotype). Benefit of concurrent use of risdiplam with other DMT therapies is unknown; however, it is being evaluated in patients previously treated with SMA-targeting therapies, including nusinersen or onasemnogene abeparvovec-xioi (JEWELFISH, types 2/3, ages 12 to 60 years). Moreover, it is evident that early diagnosis and treatment lead to better SMA outcomes, and there is a desire for treatment before symptoms appear. In 2018, the federal Recommended Uniform Screening Panel (RUSP) endorsed newborn screening for SMA. Research is ongoing for risdiplam (RAINBOWFISH), nusinersen (NURTURE), and onasemnogene abeparvovec-xioi (SPR1NT) in pre-symptomatic infants (≤ 6 weeks of age). Positive results have been reported with nusinersen and onasemnogene abeparvovec-xioi in pre-symptomatic patients, while outcomes have not been announced for risdiplam. Another oral SMN2 splicing modulator branaplam is in development for SMA type 1. Novartis plans to submit an application for branaplam to the FDA in 2023 or beyond.

FDA APPROVAL TIMELINE

May 22, 2020

✓ Breakthrough Therapy ✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$57	$166	$325	$446	$540

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

ONCOLOGY

selumetinib *oral*

AstraZeneca/Merck

PROPOSED INDICATIONS

Neurofibromatosis type 1 (NF1) and inoperable plexiform neurofibromas (PNs)

CLINICAL OVERVIEW

Neurofibromatosis (NF) is a progressive neurologic condition in which tumors, called plexiform neurofibromas (PNs), develop along the nerves throughout the body. NF is due to a mutation in the NF1 gene resulting in a deficiency of the tumor suppressor protein neurofibromin and subsequent elevation in RAS-mitogen-activated protein kinase (MAPK) activity. Selumetinib is a novel, orally administered, selective, non-ATP-competitive, MAPK kinase (MEK) 1/2 inhibitor. Selumetinib prevents tumor growth that is caused by dysregulation in the RAS/RAF/MEK/ERK signaling pathway as a result of NF1 mutations.

A pivotal phase 1, open-label clinical trial evaluated selumetinib in patients (n=24) ages 3 to 18 years with NF1 and inoperable PN. Eligible patients were either treatment-naïve to medical therapy or had received prior investigational treatment (e.g., imatinib, pirfenidone, pegylated interferon, sorafenib, and tipifarnib). All patients experienced a decrease from baseline in PN volume (median change, -31%; range, -5.8% to -47%) and 71% of patients achieved a partial response (primary endpoint). The maximum response was reached after a median of 20 cycles (range, 5 to 42 cycles). A dose of selumetinib 25 mg/m^2 twice daily was determined to be the maximum tolerated dose. Dose-limiting (grade 3) adverse effects included cellulitis, urticaria, creatinine kinase elevation, and asymptomatic decreased left ventricular ejection fraction.

The pivotal phase 2, open-label SPRINT trial enrolled patients (n=50) ages 2 to 18 years (median, 10.2 years) with NF1 and inoperable PN. Oral selumetinib 25 mg/m^2 twice-daily doses were administered continuously in 28-day cycles until disease progression or intolerable toxicity. After a median number of cycles of 19.5, the data were analyzed. Thirty-six (72%) of the patients achieved a partial response (PR), of which 22 (61%) patients experienced PR for ≥ 1 year. Stable disease was observed in an additional 24% of patients. The median change in PN volume from baseline was -27.7%. After 1 year of starting selumetinib, patients reported significant improvement in pain, motor function, and QOL.

PLACE IN THERAPY

NF1 affects 1 in 3,000 to 4,000 individuals in the US, and diagnosis is usually made in early childhood. Approximately 30% to 50% of NF1 cases involve PNs that grow along the nerves. While NF1 is usually benign, 5% to 10% of patients may develop malignant tumors of the central and peripheral nervous systems. Significant disfigurement, physical and learning disabilities, and pain are common features of the disorder. Currently, no NF-specific pharmacologic treatments exist. Surgery is often recommended to remove tumors that become symptomatic, may become cancerous, or are cosmetically unappealing; however, complete surgical removal of the tumors is rare and they typically return. For cancerous tumors, treatment may also include radiation or chemotherapy. Therapy is often provided before adolescence, when disease progression typically occurs. Long-term, dose-adjusted treatment with selumetinib resulted in partial response with no serious toxicity. If approved, selumetinib will be the first DMT approved to treat NF1 and inoperable PN. Other MEK inhibitors, mirdametinib and binimetinib (Mektovi®), are in phase 2 trials for NF.

FDA APPROVAL TIMELINE

April–May 2020

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$3	$7	$13	$19	$25

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

viltolarsen *IV*

Nippon Shinyaku

PROPOSED INDICATIONS

Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping

CLINICAL OVERVIEW

Viltolarsen is an antisense oligonucleotide that binds to mutated exon 53 genetic code allowing the abnormal code to be "skipped" during dystrophin production, creating a partially functional dystrophin protein.

A North American phase 2 study enrolled 16 male patients ages 4 to < 10 years with confirmed DMD amenable to exon 53 skipping. Patients were ambulatory and on stable doses of corticosteroids. Patients were randomized to viltolarsen 40 mg/kg (low-dose) or 80 mg/kg (high-dose) administered IV once weekly for 24 weeks. Two patients in each group were given placebo for the first 4 weeks. At week 24, both groups demonstrated a significant mean increase in percent of dystrophin protein in muscle as measured by Western Blot (low-dose cohort, 5.7% [range, 3.2-10.3]; high-dose cohort, 5.9% [range, 1.1-14.4]). Compared to a historical cohort, patients treated with viltolarsen experienced significant improvement in ambulation as measured by the time to stand from supine, climb 4 steps, and run/walk 10 meters, as well as distance walked in 6 minutes. Improvements were measured as early as 13 weeks. No safety signals were reported, and no patients discontinued viltolarsen therapy. An open-label extension trial for up to 144 weeks is ongoing. A phase 1/2 study conducted in Japan (n=16; ages 5 to 12 years) reported a statistically significant increase in mean dystrophin level with viltolarsen 80 mg/kg (3.19%; $p<0.05$) but not with the 40 mg/kg dose (1.26%; $p=0.9$).

PLACE IN THERAPY

DMD is a rare X-linked neuromuscular disorder characterized by progressive muscle degeneration and weakness. An estimated 400 to 600 boys are born with DMD each year in the US, with about 8% carrying mutations at exon 53. In DMD, gene mutations lead to a lack of functional dystrophin protein involved in maintaining muscle fiber integrity. Onset of DMD occurs between 3 to 5 years of age. Most boys affected lose the ability to walk by 12 years of age. Moreover, death due to respiratory or cardiac failure typically occurs before age 30 years. The standard of care for DMD includes systemic corticosteroids (prednisone, deflazacort [Emflaza®]) to delay progression of muscle weakness and improve respiratory function; however, side effects, such as weight gain, bone fractures, and cataracts, are associated with corticosteroid therapy.

Viltolarsen targets the exon 53 mutation and allows sections of defective genetic code to be bypassed during the dystrophin manufacturing process, creating a partially functional dystrophin protein. Sarepta was the first company to bring antisense oligonucleotides to the US market for DMD. These include eteplirsen (Exondys 51®), which targets exon 51, and golodirsen (Vyondys 53™), which targets exon 53. Golodirsen received Accelerated Approval based on surrogate data; continued approval may depend on results of confirmatory trials. Nippon Shinyaku is also seeking Accelerated Approval for viltolarsen, and full approval may also rely on post-marketing data. If approved, viltolarsen will be the second antisense oligonucleotide approved to treat DMD amenable to exon 53 skipping. Both viltolarsen and golodirsen are administered IV once weekly. The FDA denied approval of golodirsen on initial submission due to 2 concerns: risk of IV infusion port infection and renal toxicity, neither of which have been reported with viltolarsen. Sarepta's antisense oligonucleotide IV casimersen that targets exon 45 mutations is also in late-stage development for DMD.

FDA APPROVAL TIMELINE

October 2, 2020

✓ Fast Track ✓ Orphan Drug ✓ Rare Pediatric Disease

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$43	$99	$131	$142	$148

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, and 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' ability 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 and 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 on March 23, 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." Some states, however, 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 26 biosimilars have received FDA approval. Of these, only 13 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)	Samsung Bioepis/ 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)
Abrilada™ (adalimumab-afzb)	Pfizer	November 2019	-	Humira (Abbvie)

APPROVED BIOSIMILARS *continued*				
Brand Name (Nonproprietary name)	**Manufacturer**	**Approval Date**	**Commercially Available**	**Originator Product (Manufacturer)**
Ziextenzo® (pegfilgrastim-bmez)	Novartis/Sandoz	November 2019	✓	Neulasta (Amgen)
Avsola™ (infliximab-axxq)	Amgen	December 2019	-	Remicade (Janssen)

* 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.

ONCOLOGY

bevacizumab (SB8) *IV*

Merck/Samsung Bioepis

SB8 is a biosimilar to Genentech's Avastin, a VEGF-specific angiogenesis inhibitor indicated for the treatment of metastatic colorectal cancer, non-squamous non-small cell lung cancer (nsNSCLC), glioblastoma, metastatic renal cell carcinoma (RCC), and recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer.

FDA APPROVAL TIMELINE

July–September 2020

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$2,316	$1,736	$1,420	$1,250	$1,081

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 who are receiving myelosuppressive anti-cancer drugs; following induction or consolidation chemotherapy for acute myeloid leukemia (AML); with nonmyeloid malignancies in patients who are 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 in patients who are 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)

2020	2021	2022	2023	2024
$119	$101	$88	$79	$73

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

BLOOD MODIFIER

pegfilgrastim *SC*

Lapelga and PF-06881894 are biosimilars to Amgen's Neulasta, a leukocyte growth factor indicated for use in patients with nonmyeloid malignancies who are receiving myelosuppressive anti-cancer drugs and in patients acutely exposed to myelosuppressive doses of radiation (HSARS).

FDA APPROVAL TIMELINE

Apotex (Lapelga)
Pending

Pfizer/Hospira (PF-06881894)
June 2020

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$2,033	$1,673	$1,376	$1,158	$1,006

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

OPHTHALMOLOGY

ranibizumab (FYB201)

Coherus

FYB201 is an investigational biosimilar to Genentech's Lucentis®, a VEGF inhibitor indicated for the treatment of neovascular (wet) age-related macular degeneration (AMD), macular edema following retinal vein occlusion (RVO), diabetic macular edema (DME), diabetic retinopathy, and myopic choroidal neovascularization.

FDA APPROVAL TIMELINE

December 2020

FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$1,696	$1,467	$1,309	$1,170	$999

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

ONCOLOGY

rituximab (ABP-798)

Amgen/Allergan

ABP-798 is an investigational biosimilar to Genentech's Rituxan, a CD20-directed cytolytic antibody indicated for the treatment of non-Hodgkin's lymphoma (NHL), chronic lymphocytic leukemia (CLL), rheumatoid arthritis (RA), and antineutrophil cytoplasmic antibodies (ANCA)-associated vasculitis.

FDA APPROVAL TIMELINE

October 19, 2020

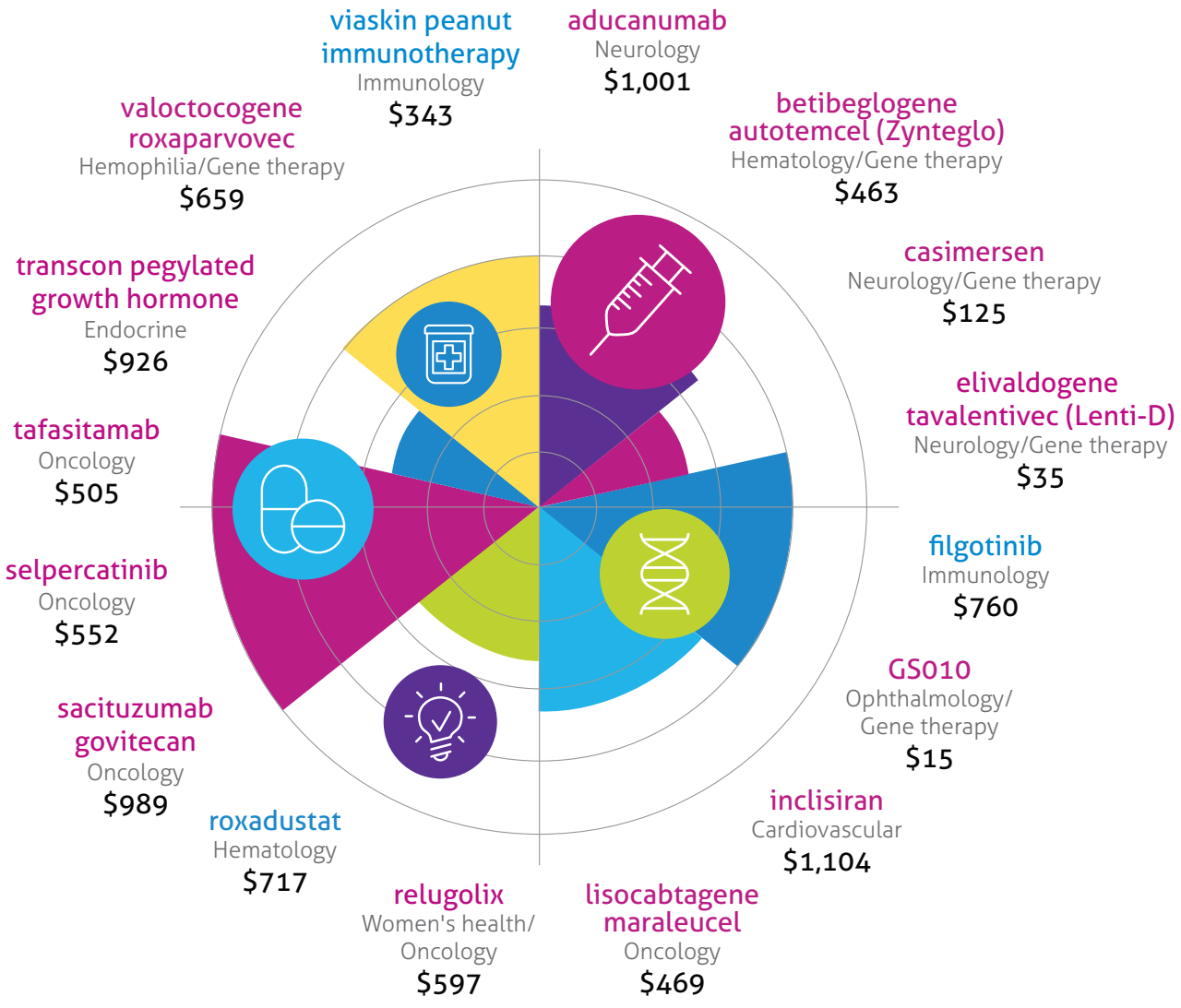
FINANCIAL FORECAST (reported in millions)

2020	2021	2022	2023	2024
$3,447	$2,513	$2,018	$1,732	$1,499

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 2024, are displayed. The financials are projected total annual US sales, reported in *millions.*

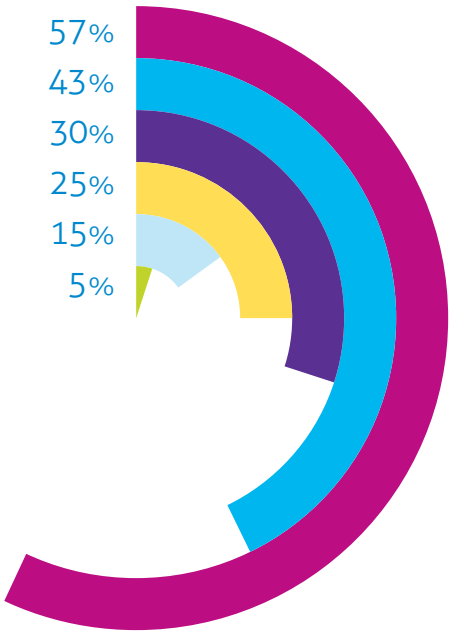


★ Specialty drug names appear in magenta throughout the publication.

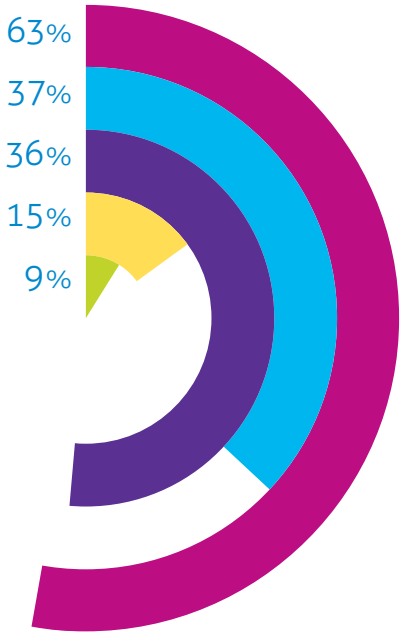
PIPELINE **DRUG LIST**

The pipeline drug list is an aerial outline of drugs with anticipated FDA approval through 2021. 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
dulaglutide (Trulicity®)	Eli Lilly	T2DM CV outcomes	SC	Submitted – sNDA	January 2020
peanut protein capsule (AR101)	Aimmune	Peanut allergy (children and adolescents)	Oral	Submitted – BLA; Breakthrough Therapy; Fast Track	January 2020
durvalumab (Imfinzi®)	AstraZeneca	SCLC (1st-line, extensive-disease)	IV	Submitted – sBLA; Orphan Drug; Priority Review	Jan–Mar 2020
osilodrostat	Novartis	Cushing's syndrome	Oral	Submitted – NDA; Orphan Drug	Jan–Mar 2020
rimegepant	Biohaven	Migraine treatment	Oral	Submitted – NDA; Priority Review (ODT only)	Late February 2020
paclitaxel injection concentrate for suspension	Sun Advanced Research	Breast cancer	IV	Submitted – 505(b)(2) NDA	Feb–Mar 2020
empagliflozin (Jardiance®)	Boehringer Ingelheim	T1DM	Oral	Submitted – sNDA	Feb–May 2020
ethinyl estradiol/ levonorgestrel	Agile	Contraception	Transdermal	Submitted – 505(b)(2) NDA	02/16/2020
pembrolizumab (Keytruda®) - 6-week dosing regimen	Merck	Melanoma; Classical Hodgkin lymphoma; Primary mediastinal large B cell lymphoma; Gastric cancer; HCC; Merkel cell carcinoma	IV	Submitted – sBLA; Breakthrough Therapy; Orphan Drug	02/18/2020
meloxicam	Recro	Postsurgical pain	IV	Submitted – 505(b)(2) NDA	02/20/2020
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
naloxone nasal spray	Insys	Substance use disorder	Intranasal	Submitted – 505(b)(2) NDA	March 2020
atezolizumab (Tecentriq®)	Genentech	HCC (1st-line, unresectable, in combination with bevacizumab)	IV	Submitted – sBLA; Breakthrough Therapy; RTOR	Mar–Apr 2020
bimatoprost SR	Allergan	Glaucoma/ocular hypertension	Intraocular	Submitted – NDA	Mar–Apr 2020
celecoxib/tramadol	Mundipharma	Moderate to severe pain	Oral	Submitted – NDA	Mar–Apr 2020
cysteamine DR oral granule (Procysbi®)	Horizon	Nephropathic cystinosis	Oral	Submitted – sNDA	Mar–Apr 2020
exenatide SC pump	Intarcia/Janssen	T2DM	SC	Submitted – NDA	03/09/2020
nivolumab (Opdivo®)	Bristol-Myers Squibb	HCC (previously treated with sorafenib, in combination with ipilimumab)	IV	Submitted – sBLA; Breakthrough Therapy; Orphan Drug; Priority Review	03/10/2020

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	03/17/2020
fenfluramine (low dose)	Zogenix	Dravet syndrome	Oral	Submitted – NDA; Fast Track; Orphan Drug; Priority Review	03/25/2020
ozanimod	Celgene	MS (relapsing)	Oral	Submitted – NDA	03/25/2020
bupivacaine/meloxicam ER	Heron	Postsurgical pain	Surgical site instillation	Submitted – NDA; Breakthrough Therapy; Fast Track	03/26/2020
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
binimetinib (Mektovi)	Pfizer	CRC (BRAF V600E-mutant, metastatic, in combination with encorafenib and cetuximab)	Oral	Submitted – sNDA; Breakthrough Therapy	April 2020
cetuximab (Erbitux®)	Eli Lilly	CRC (BRAF V600E-mutant, 2nd- or 3rd-line, in combination with encorafenib ± binimetinib)	IV	Submitted – sBLA; Breakthrough Therapy; Priority Review	April 2020
encorafenib (Braftovi®)	Pfizer	CRC (BRAF V600E-mutant, metastatic, in combination with cetuximab ± binimetinib)	Oral	Submitted – sNDA; Breakthrough Therapy; Priority Review	April 2020
neratinib (Nerlynx®)	Puma	Breast cancer (HER2+, metastatic, in combination with capecitabine, 3rd-line)	Oral	Submitted – sNDA	April 2020
dapagliflozin (Farxiga®)	AstraZeneca	T2DM-related CV risk reduction in patients with heart failure	Oral	Submitted – sNDA; Fast Track; Priority Review	Apr–Jun 2020
mannitol dry powder	Pharmaxis	CF (adults)	Inhaled	Submitted – NDA; Fast Track; Orphan Drug	Apr–Jun 2020
olaparib (Lynparza®)	AstraZeneca	Ovarian cancer (maintenance, post first-line platinum-based chemotherapy and bevacizumab)	Oral	Submitted – sNDA; Orphan Drug; Priority Review	Apr–Jun 2020
satralizumab	Genentech	Neuromyelitis optica (Devic's syndrome)	SC	Submitted – BLA; Breakthrough Therapy; Orphan Drug	Apr–May 2020
selumetinib	AstraZeneca/Merck	Neurofibromatosis (NF) and inoperable plexiform neurofibromas (PN)	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug; Priority Review	Apr–May 2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
luspatercept-aamt (Reblozyl®)	Acceleron	Myelodysplastic syndrome	SC	Submitted – sBLA; Fast Track; Orphan Drug	04/03/2020
remimazolam	Cosmo	Anesthesia	IV	Submitted – NDA	04/03/2020
mitomycin	Urogen	Bladder cancer (low-grade, upper tract)	Intravesical	Submitted – 505(b)(2) NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	04/17/2020
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
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis (non-radiographic)	SC	Submitted – sBLA	May–Jun 2020
nadofaragene firadenovec	FKD	Bladder cancer (high-grade, Bacillus Calmette-Guerin [BCG]-unresponsive, non-muscle invasive)	Intravesical	Submitted – BLA; Breakthrough Therapy; Fast Track; Priority Review	May–Jun 2020
ticagrelor (Brilinta®)	AstraZeneca	Coronary artery disease in patients with T2DM	Oral	Submitted – sNDA	May–Aug 2020
dasotraline	Sumitomo Dainippon	Binge eating disorder	Oral	Submitted – NDA	05/14/2020
nivolumab (Opdivo)	Bristol-Myers Squibb	NSCLC (metastatic, recurrent, EGFR-negative, ALK-negative)	IV	Submitted – sBLA; Fast Track; Priority Review	05/15/2020
rucaparib (Rubraca®)	Clovis Oncology	Prostate cancer	Oral	Submitted – sNDA; Breakthrough Therapy; Priority Review	05/15/2020
apomorphine	Sumitomo Dainippon	Parkinson's disease ("off" episodes)	SL/Oral transmucosal	Submitted – 505(b)(2) NDA; Fast Track	05/21/2020
risdiplam	Genentech	Spinal muscular atrophy (types 1, 2, 3)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	05/22/2020
artesunate	La Jolla	Malaria (severe)	Not specified	Submitted – NDA; Breakthrough Therapy; Orphan Drug	05/25/2020
dupilumab (Dupixent®)	Sanofi	Atopic dermatitis (ages 6 to 11 years)	SC	Submitted – sBLA; Breakthrough Therapy; Priority Review	05/26/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
pemigatinib	Incyte	Biliary tract cancer (FGFR2 translocated, 2nd-line)	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug; Priority Review	05/30/2020
pegfilgrastim (biosimilar to Amgen's Neulasta)	Pfizer/Hospira	Neutropenia/leukopenia	SC	Submitted – BLA	June 2020
abicipar pegol	Allergan	Wet AMD	Intraocular	Submitted – BLA	Jun–Jul 2020
inebilizumab	Viela Bio	Neuromyelitis optica (Devic's syndrome)	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug	Jun–Jul 2020
selpercatinib	Eli Lilly	NSCLC (RET-altered); Thyroid cancer (RET-altered)	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug	Jun–Aug 2020
diazepam film	Aquestive	Seizure clusters	Oral transmucosal	Submitted – 505(b)(2) NDA; Fast Track; Orphan Drug	06/02/2020
minocycline 1.5% foam	Foamix	Rosacea	Topical	Submitted – 505(b)(2) NDA	06/02/2020
sacituzumab govitecan	Immunomedics	Breast cancer (triple negative, metastatic, ≥ 2 prior therapies)	IV	Submitted – BLA; Breakthrough Therapy; Fast Track	06/02/2020
elagolix (Orilissa®)	Abbvie	Uterine fibroids	Oral	Submitted – sNDA	06/05/2020
ketotifen	Bausch Health	Allergic conjunctivitis	Ophthalmic	Submitted – 505(b)(2) NDA	06/11/2020
fosfomycin	Nabriva	UTI (complicated)	IV	Submitted – 505(b)(2) NDA; Fast Track; QIDP	06/19/2020
metoclopramide	Evoke	Diabetic gastroparesis (in women)	Oral	Submitted – sNDA; Breakthrough Therapy; Priority Review	06/19/2020
obeticholic acid (Ocaliva)	Intercept	NASH	Intranasal	Submitted – 505(b)(2) NDA	06/26/2020
octreotide	Chiasma	Acromegaly	Oral	Submitted – 505(b)(2) NDA; Orphan Drug	06/26/2020
pembrolizumab (Keytruda)	Merck	Cutaneous squamous cell carcinoma (recurrent/ metastatic, not curable with surgery/radiation)	IV	Submitted – sBLA	06/29/2020
nintedanib (Ofev®)	Boehringer Ingelheim	Pulmonary fibrosis	Oral	Submitted – sNDA; Breakthrough Therapy	July 2020
insulin lispro, ultra rapid	Eli Lilly	T1DM; T2DM	SC	Submitted – NDA	Jul–Oct 2020
bevacizumab (biosimilar to Genentech's Avastin)	Merck/Samsung Bioepis	CRC; NSCLC; Glioblastoma; RCC; Ovarian cancer	IV	Submitted – BLA	Jul–Sep 2020
omalizumab (Xolair®)	Genentech	Nasal polyposis	SC	Submitted – sBLA	Jul–Sep 2020
dantrolene	Eagle	Heat stroke (exertional)	IV	Submitted – sNDA; Fast Track; Orphan Drug	07/09/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
daratumumab (Darzalex®)	Janssen	Multiple myeloma	SC	Submitted – sBLA	07/10/2020
cantharidin 0.7% solution	Verrica	Molluscum contagiosum	Topical	Submitted – NDA	07/13/2020
guselkumab (Tremfya®)	Janssen	PsA	SC	Submitted – sBLA	07/16/2020
oxymetazoline 0.1% solution	Osmotica	Acquired blepharoptosis	Ophthalmic	Submitted – NDA	07/16/2020
capsaicin (Qutenza®)	Grünenthal	Diabetic peripheral neuropathy	Topical	Submitted – sNDA	07/19/2020
calcipotriene/ betamethasone dipropionate cream	MC2	PSO	Topical	Submitted – 505(b)(2) NDA	07/20/2020
sodium oxybate (low dose)	Jazz	Narcolepsy	Oral	Submitted – NDA; Priority Review	07/21/2020
donepezil	Corium	Alzheimer's disease	Transdermal	Submitted – NDA	07/30/2020
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
capmatinib	Novartis	NSCLC	Oral	Submitted – NDA; Breakthrough Therapy; Orphan Drug	August 2020
esketamine (Spravato®)	Janssen	MDD (with suicidal ideation with intent)	Intranasal	Submitted – sNDA; Breakthrough Therapy; Fast Track	08/02/2020
fostemsavir	Viiv	HIV-1 infection (multidrug-resistant)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track	08/05/2020
viaskin peanut immunotherapy	DBV	Peanut allergy (ages 4 to 11 years)	Transdermal	Submitted – BLA; Breakthrough Therapy; Fast Track	08/05/2020
ustekinumab (Stelara®)	Janssen	PSO (ages 6 to 11 years)	IV, SC	Submitted – sBLA	08/07/2020
berotralstat	Biocryst	Hereditary angioedema	Oral	Submitted – NDA; Fast Track; Orphan Drug	08/11/2020
KTE-X19	Gilead	Mantle cell lymphoma (relapsed/refractory)	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug	08/11/2020
belantamab mafodotin	GlaxoSmithKline	Multiple myeloma (relapsed/refractory)	SC	Submitted – BLA; Breakthrough Therapy; Orphan Drug; Priority Review	08/14/2020
dolutegravir/lamivudine (Dovato®)	GlaxoSmithKline	HIV-1 treatment (switch therapy in virologically suppressed adults)	Oral	Submitted – sNDA	08/16/2020
ripretinib	Deciphera	Gastrointestinal stromal tumor (prior treatment with imatinib, sunitinib, and regorafenib)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug; RTOR	08/16/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
lisocabtagene maraleucel	Celgene	DLBCL (relapsed/ refractory)	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug; RMAT	08/18/2020
filgotinib	Gilead	RA	Oral	Submitted – NDA; Priority Review	08/19/2020
margetuximab	Macrogenics	Breast cancer (HER2+, in combination with chemotherapy)	IV	Submitted – BLA; Fast Track	08/19/2020
veverimer	Tricida	CKD-related metabolic acidosis	Oral	Submitted – NDA	08/22/2020
clascoterone	Cassiopea	Acne	Topical	Submitted – NDA	08/27/2020
tafasitamab	Morphosys	DLBCL (relasped/ refractory, post-lenalidomide monotherapy)	IV	Submitted – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	08/28/2020
HIV vaccine (Remune)	Immune Response Biopharma	HIV infection treatment (pediatrics)	IM	Submitted – BLA; Orphan Drug	Sep–Dec 2020
ibrutinib (Imbruvica®)	Abbvie	CLL/SLL (1st-line, ages < 70 years, in combination with rituximab)	Oral	Submitted – sNDA; Orphan Drug; RTOR	09/08/2020
somapacitan	Novo Nordisk	Growth hormone deficiency (adults)	SC	Submitted – BLA	09/21/2020
linaclotide acetate (Linzess®)	Ironwood	IBS (treatment of abdominal symptoms)	Oral	Submitted – sNDA	October 2020
selinexor (Xpovio®)	Karyopharm	DLBCL (relapsed/ refractory, post ≥ 2 multi-drug therapies, SCT-ineligible)	Oral	Submitted – sNDA; Fast Track; Orphan Drug	October 2020
lurbinectedin	Jazz	SCLC (relapsed)	IV	Submitted – NDA; Orphan Drug	Oct–Dec 2020
tazemetostat (Tazverik™)	Epizyme	Follicular lymphoma (relapsed/refractory, ≥ 2 prior therapies)	Oral	Submitted – sNDA; Fast Track; Orphan Drug	Oct–Dec 2020
tucatinib	Seattle Genetics	Breast cancer (locally advanced unresectable, metastatic [including brain], HER2+, in combination with trastuzumab and capecitabine)	Oral	Submitted – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	Oct–Dec 2020
valoctocogene roxaparvovec	Biomarin	Hemophilia A	IV	Submitted – BLA; Breakthrough Therapy; Orphan Drug	Oct–Dec 2020
hydrocortisone granules	Diurnal	Congenital adrenal hyperplasia (pediatrics)	Oral	Submitted – 505(b)(2) NDA; Orphan Drug	10/02/2020
viltolarsen	Nippon Shinyaku	DMD (exon 53 skipping)	IV	Submitted – NDA; Fast Track; Orphan Drug; Rare Pediatric Disease	10/02/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
tramadol	Fortress	Pain (moderate to severe, medically supervised setting)	IV	Submitted – 505(b)(2) NDA	10/09/2020
dolutegravir (Tivicay®) dispersible tablet	GlaxoSmithKline	HIV-1 treatment (pediatrics)	Oral	Submitted – sNDA	10/13/2020
burosumab-twza (Crysvita®)	Ultrenyx	Tumor-induced osteomalacia	IV, SC	Submitted – sBLA	10/16/2020
rituximab (biosimilar to Genentech's Rituxan)	Amgen/Allergan	RA; CLL/SLL; NHL (indolent); ANCA-associated vasculitis	IV	Submitted – BLA	10/19/2020
zolmitriptan (micro-needle patch)	Zosano	Migraine treatment	Transdermal	Submitted – 505(b)(2) NDA	10/20/2020
eflapegrastim	Spectrum	Neutropenia/leukopenia	SC	Submitted – BLA	10/23/2020
viloxazine	Supernus	ADHD	Oral	Submitted – NDA	11/06/2020
samidorphan/olanzapine	Alkermes	Bipolar disorder; Schizophrenia	Oral	Submitted – NDA	11/15/2020
treprostinil dry powder	GlaxoSmithKline	PAH	Inhaled	Submitted – 505(b)(2) NDA	11/27/2020
inclisiran	The Medicines Company	Dyslipidemia (in secondary prevention patients with ASCVD and familial hypercholesterolemia)	SC	Submitted – NDA; Orphan Drug	December 2020
ranibizumab (biosimilar to Genentech's Lucentis)	Coherus	Wet AMD	Intraocular	Submitted – BLA	December 2020
roxadustat	AstraZeneca	Anemia due to CKD (dialysis-independent; dialysis-dependent)	Oral	Submitted – NDA	12/23/2020
ansofaxine	Luye	MDD	Oral	Submitted – NDA	12/25/2020
vibegron	Urovant	Overactive bladder	Oral	Submitted – NDA	12/30/2020
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
oxycodone ER	Intelliceutics	Chronic pain	Oral	Submitted – 505(b)(2) NDA; Fast Track	Pending
5-aminolevulinic acid (Ameluz®)	Biofrontera	Actinic keratoses (with conventional photodynamic therapy)	Topical	Phase 3 – sNDA	TBD
abaloparatide-TD	Radius	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
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 – BLA	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	UC (pouchitis)	Rectal	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
alpha 1 proteinase inhibitor	Kamada	Emphysema	Inhaled	Phase 3 – BLA; 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
APR-246	Aprea	Myelodysplastic syndrome	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
arimoclomol	Orphazyme	Niemann-Pick disease	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
asciminib	Novartis	Chronic myelogenous leukemia (CML)	Oral	Phase 3 – NDA; Orphan Drug	TBD
ataluren (Translarna®)	PTC	DMD	Oral	Phase 3 – sNDA; Fast Track; Orphan Drug	TBD
atezolizumab (Tecentriq)	Genentech	CRC; RCC; Melanoma; Ovarian cancer; NSCLC (nonsquamous, 1st-line); NSCLC (neo-adjuvant); Bladder cancer (1st-line metastatic); Breast cancer (adjuvant, TNBC); Breast cancer (1st-line)	IV	Phase 3 – sBLA; 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
avatrombopag (Doptelet®)	Ararx	Thrombocytopenia (chemotherapy-induced)	Oral	Phase 3 – sNDA; Orphan Drug	TBD
azacitidine	Celgene	AML	Oral	Phase 3 – NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
baclofen/naltrexone/ sorbitol	Pharnext	Charcot-Marie-Tooth disease	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
baricitinib (Olumiant®)	Eli Lilly	Atopic dermatitis; SLE	Oral	Phase 3 – sNDA; Fast Track	TBD
bedaquiline (Sirturo®)	Janssen	Tuberculosis (pediatric patients with pulmonary multidrug-resistant tuberculosis (MDR-TB)	Oral	Phase 3 – sNDA; Fast Track; Orphan Drug	TBD
belimumab (Benlysta®)	GlaxoSmithKline	Lupus nephritis	IV	Phase 3 – sBLA	TBD
benralizumab (Fasenra®)	AstraZeneca	Nasal polyposis	SC	Phase 3 – sBLA	TBD
betibeglogene autotemcel (Zynteglo)	Bluebird Bio	Thalassemia	IV	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	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
bimekizumab	UCB	Axial spondyloarthritis; PSO; PsA	IV	Phase 3 – BLA	TBD
biotin (high dose)	Medday	MS	Oral	Phase 3 – NDA	TBD
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease-related agitation; 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
cabotegravir (long-acting)	Viiv	HIV-1 infection preexposure prevention (PrEP)	IM	Phase 3 – NDA	TBD
cabozantinib (Cabometyx®)	Exelixis	RCC (1st-line, in combination with nivolumab)	Oral	Phase 3 – sNDA; Breakthrough Therapy; Fast Track	TBD
calmangafodipir	Pledpharma	Chemotherapy-induced peripheral neuropathy	IV	Phase 3 – NDA	TBD
cannabidiol (Epidiolex)	GW	Rett syndrome; Tuberous sclerosis complex	Oral	Phase 3 – sNDA; Orphan Drug	TBD
cannabidiol gel	Zynerba	Fragile X syndrome	Topical	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
capsaicin	Centrexion	Osteoarthritis	Intraarticular	Phase 3 – NDA; Fast Track	TBD
carglumic acid	Recordati	Hyperammonemia (genetic autosomal disorder-related)	Oral	Phase 3 – NDA; Orphan Drug	TBD
casimersen	Sarepta	DMD	IV	Phase 3 – NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
cedazuridine/decitabine	Otsuka	AML; Chronic myelomonocytic leukemia (CMML); Myelodysplastic syndrome	Oral	Phase 3 – NDA; Orphan Drug	TBD
cediranib	AstraZeneca	Ovarian cancer; Biliary tract cancer	Oral	Phase 3 – NDA; Orphan Drug	TBD
cefiderocol (Fetroja®)	Shionogi	HAP	IV	Phase 3 – sNDA	TBD
ceftobiprole medocaril	Basilea	ABSSSI	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
conbercept	Chengdu Kanghong	Wet AMD	Intraocular	Phase 3 – BLA	TBD
concizumab	Novo Nordisk	Hemophilia A and B	IV, SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
cortrophin (purified gel)	ANI	MS	IV	Phase 3 – sNDA	TBD
crisantaspase	Jazz	ALL	IM, IV	Phase 3 – BLA; Fast Track	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
daratumumab-rHuPH20	Janssen	Amyloidosis	SC	Phase 3 – BLA	TBD
dasiglucagon	Zealand	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 – NDA; Orphan Drug	TBD
dehydrated human amnion-chorion membrane	Mimedx	Plantar fasciitis	Pedal injection	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
dexmedetomidine	Bioxcel	Schizophrenia	SL/Oral transmucosal	Phase 3 – NDA; Fast Track	TBD
dianhydrogalactitol	Delmar	Brain cancer	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
difelikefalin	Enteris	Pruritus (hemodialysis-related)	IV	Phase 3 – NDA; Breakthrough Therapy	TBD
digoxin immune Fab	AMAG	Eclampsia/pre-eclampsia	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
docosahexaenoic acid	Micelle	Sickle cell disease	Oral	Phase 3 – NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
donaperminogene seltoplasmid	Helixmith	Diabetic peripheral neuropathy; Diabetic foot ulcers; Peripheral arterial disease	IM	Phase 3 – BLA; RMAT	TBD
dovitinib lactate	Oncology Venture	RCC	Oral	Phase 3 – NDA	TBD
dupilumab (Dupixent)	Sanofi	COPD; Esophagitis	SC	Phase 3 – sBLA	TBD
durvalumab (Imfinzi)	AstraZeneca	Bladder cancer (in combination with tremelimumab); NSCLC (1st-line, in combination with tremelimumab)	IV	Phase 3 – sBLA; Breakthrough Therapy; Fast Track	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 – NDA; Fast Track	TBD
dust mite immunotherapy	Stallergenes	Allergic rhinitis	SL/Oral transmucosal	Phase 3 – BLA	TBD
edasalonexent	Catabasis	DMD	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
efgartigimod	Argenx	Myasthenia gravis; Immune thrombocytopenic purpura	IV, SC	Phase 3 – BLA; Orphan Drug	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)	N/A	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	Mithra	Menopausal vasomotor symptoms	Oral	Phase 3 – NDA	TBD
estetrol/drospirenone	Mayne	Contraception	Oral	Phase 3 – NDA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Coherus	RA; JIA; AS; PSO; PsA	SC	Phase 3 – BLA	TBD
etranacogene dezaparvovec	Uniqure	Hemophilia B	IV	Phase 3 – BLA; Breakthrough Therapy	TBD
etrasimod	Arena	UC	Oral	Phase 3 – NDA	TBD
etrolizumab	Genentech	CD; UC	IV, SC	Phase 3 – BLA; Orphan Drug	TBD
evinacumab	Regeneron	Dyslipidemia/ hypercholesterolemia	SC	Phase 3 – BLA; Breakthrough Therapy	TBD
fasinumab	Regeneron	Osteoarthritis	IV, SC	Phase 3 – BLA	TBD
fenfluramine (low dose)	Zogenix	Lennox-Gastaut syndrome	Oral	Phase 3 – NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
fexapotide triflutate	Nymox	Benign prostatic hyperplasia	Intratumoral	Phase 3 – NDA	TBD
fezolinetant	Astellas	Menopause vasomotor symptoms	Oral	Phase 3 – NDA	TBD
filgotinib	Gilead	PsA; 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	Female infertility	SC	Phase 3 – 505(b)(2) NDA	TBD
follitropin alfa (follow-on to EMD Serono's Gonal-F)	Finox	Female infertility	SC	Phase 3 – 505(b)(2) NDA	TBD
follitropin delta	Ferring	Female infertility	IV	Phase 3 – BLA	TBD
fusidic acid	Arrevus	ABSSSI	Oral	Phase 3 – NDA; Orphan Drug; QIDP	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 – NDA	TBD
gepotidacin	GlaxoSmithKline	Uncomplicated UTI	Oral	Phase 3 – NDA; QIDP	TBD
givinostat	Italfarmaco	DMD	Oral	Phase 3 – NDA; Orphan Drug	TBD
glatiramer acetate depot	Mylan	MS	IM	Phase 3 – 505(b)(2) NDA	TBD
GLPG1690	Galapos	Idiopathic pulmonary fibrosis	Oral	Phase 3 – NDA; Orphan Drug	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
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
iclaprim	Motif Bio	ABSSSI; HAP	IV	Phase 3 – NDA; Fast Track; QIDP	TBD
idasanutlin	Roche	AML	Oral	Phase 3 – NDA	TBD
idebenone	Santhera	DMD	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
idecabtagene vicluecel	Celgene	Multiple myeloma	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
idursulfase	Takeda	Mucopolysaccharidosis II (Hunter syndrome)	Intrathecal	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
immunoglobulin IV 10%	Prometic Life Sciences	Primary immunodeficiencies	IV	Phase 3 – BLA	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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	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; Fast Track	TBD
leriglitazone	Minoryx	Adrenomyeloneuropathy (adrenoleukodystrophy)	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
leronlimab	Cytodyn	HIV-1 infection treatment	IV, 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
linzagolix	ObsEva	Endometriosis; Uterine fibroids	Oral	Phase 3 – NDA	TBD
L-lactic acid/citric acid/ potassium bitartrate	Evofem	Contraception; UTI	Intravaginal	Phase 3 – NDA; Fast Track; QIDP	TBD
lonafarnib	Eiger	Hepatitis D infection; Progeria	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
lorecivivint	Samumed	Osteoarthritis (knee)	Intraarticular	Phase 3 – NDA	TBD
lumasiran	Alnylam	Hyperoxaluria	SC	Phase 3 – NDA; Breakthrough Therapy; Orphan Drug	TBD
lumateperone (Caplyta®)	Intra-Cellular Therapies	Bipolar disorder	Oral	Phase 3 – sNDA	TBD
lutetium 177Lu-PSMA-617	Novartis	Prostate cancer	IV	Phase 3 – NDA	TBD
maribavir	Takeda	Cytomegalovirus infection treatment	Oral	Phase 3 – NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
marstacimab	Pfizer	Hemophilia A and B	IV, SC	Phase 3 – BLA; Orphan Drug	TBD
masitinib mesylate	AB Science	Asthma	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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	*C. difficile* infection (recurrent)	Rectal	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
mirikizumab	Eli Lilly	PSO; CD; UC	IV, SC	Phase 3 – BLA	TBD
mirvetuximab soravtansine	Immunogen	Ovarian cancer	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
molgramostim	Savara	Pulmonary alveolar proteinosis	Inhaled	Phase 3 – BLA; Breakthrough Therapy; Fast Track; Orphan Drug	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
narsoplimab	Omeros	HSCT-associated thrombotic microangiopathy	IV, SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
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	RSV infection prevention	N/A	Phase 3 – BLA; Breakthrough Therapy; Fast Track	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 – NDA	TBD
nitric oxide	Novan	Molluscum contagiosum	Topical	Phase 3 – NDA	TBD
olaparib (Lynparza)	AstraZeneca	Breast cancer (metastatic, adjuvant treatment); Ovarian cancer (germline BRCA-mutated, platinum-sensitive relapsed); Ovarian cancer (2nd-line, in combination with cediranib)	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	Peanut allergy	SC	Phase 3 – sBLA; Breakthrough Therapy	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
omidubicel	Gamida Cell	Hematologic cancer	N/A	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	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 Bio	Bladder cancer (BCG-unresponsive, non-muscle invasive)	Intravesical	Phase 3 – BLA; Fast Track	TBD
oxalobacter formigenes	Oxthera	Hyperoxaluria	Oral	Phase 3 – BLA; Orphan Drug	TBD
ozanimod	Celgene	CD; UC	Oral	Phase 3 – NDA	TBD
paliperidone (6-month injectable)	Janssen	Schizophrenia	IM	Phase 3 – NDA	TBD
pamrevlumab	Fibrogen	Idiopathic pulmonary fibrosis	IV	Phase 3 – BLA; Fast Track; Orphan Drug	TBD
pegcetacoplan	Apellis	Paroxysmal nocturnal hemoglobinuria	SC	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
pegunigalsidase alfa	Chiesi	Fabry's disease	IV	Phase 3 – BLA; Fast Track	TBD
pertuzumab (Perjeta®)	Genentech	Breast cancer (HER2+, adjuvant, in combination with ado-trastuzumab)	IV	Phase 3 – sBLA	TBD
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
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
prasterone (Intrarosa®)	AMAG	Female sexual arousal disorder	Intravaginal	Phase 3 – sNDA	TBD
ranibizumab intravitreal implant	Genentech	Wet AMD	Intraocular	Phase 3 – BLA	TBD
ranibizumab (biosimilar to Genentech's Lucentis)	Samsung Bioepis	Wet AMD	Intraocular	Phase 3 – BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ranibizumab (biosimilar to Genentech's Lucentis)	Stada Arzneimittel	Wet AMD	Intraocular	Phase 3 – BLA	TBD
ravulizumab-cwvz (Ultomiris®)	Alexion	Paroxysmal nocturnal hemoglobinuria	SC	Phase 3 – sBLA; Orphan Drug	TBD
REGN-EB3	Regeneron	Ebola virus infection treatment	IV	Phase 2 – BLA; Breakthrough Therapy; Orphan Drug	TBD
relebactam/imipenem/cilastatin	Merck	HAP	IV	Phase 3 – NDA; Fast Track; QIDP	TBD
relugolix	Myovant	Prostate cancer; Endometriosis; Uterine fibroids	Oral	Phase 3 – NDA	TBD
remestemcel-L	Mesoblast	GVHD (steroid-refractory, pediatric)	IV, SC	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; Fast Track	TBD
ridinilazole	Summit	*C. difficile*-associated diarrhea	Oral	Phase 3 – NDA; Fast Track; QIDP	TBD
risankizumab-rzaa (Skyrizi®)	Abbvie	CD; UC; PsA	IV, SC	Phase 3 – sBLA; Orphan Drug	TBD
rituximab (biosimilar to Genentech's Rituxan)	Archigen	RA; CLL/SLL; NHL (indolent); ANCA-associated vasculitis	IV, SC	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 oncology treatment	Oral	Phase 3 – NDA	TBD
RSV nanoparticle vaccine	Novavax	RSV infection prevention	IM	Phase 3 – BLA; Fast Track	TBD
ruxolitinib (Jakafi®)	Incyte	GVHD (steroid-refractory)	Oral	Phase 3 – sNDA; Breakthrough Therapy; Orphan Drug	TBD
ruxolitinib cream	Incyte	Atopic dermatitis; Vitiligo	Topical	Phase 3 – NDA	TBD
sacubitril/valsartan (Entresto®)	Novartis	Post-acute myocardial infarction	Oral	Phase 3 – sNDA; Fast Track	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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	Diabetic nephropathy; Obesity	SC	Phase 3 – sNDA	TBD
serlopitant	Menlo	Pruritus (prurigo nodularis-related)	Oral	Phase 3 – NDA; Breakthrough Therapy	TBD
setmelanotide	Rhythm	Obesity (proopiomelanocortin and leptin receptor deficiency)	SC	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD
sodium hyaluronate/ triamcinolone hexacetonide	Anika	Osteoarthritis (knee)	Intraarticular	Phase 3 – NDA	TBD
sodium oxybate (once-nightly dosing)	Avadel	Narcolepsy	Oral	Phase 3 – 505(b)(2) NDA; 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
spartalizumab	Novartis	Melanoma	IV	Phase 3 – BLA	TBD
sulopenem	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	DMD	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
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
teplizumab	Provention	T1DM	IV	Phase 3 – BLA; Breakthrough Therapy; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
teprasiran	Quark	Delayed graft function; Kidney injury prevention following cardiac surgery	IV	Phase 3 – NDA	TBD
teriflunomide	Sanofi	MS (pediatrics)	Oral	Phase 3 – sBLA	TBD
terlipressin	Mallinckrodt	Hepatorenal syndrome	IV	Phase 3 – NDA; Fast Track; Orphan Drug	TBD
tezepelumab	AstraZeneca	Asthma	IV, SC	Phase 3 – BLA; Breakthrough Therapy	TBD
timbetasin	Regenerx	Dry eye syndrome	Topical	Phase 3 – NDA	TBD
tirbanibulin	Almirall	Actinic keratoses	Oral, Topical	Phase 3 – NDA	TBD
tirzepatide	Eli Lilly	T1DM	SC	Phase 3 – NDA	TBD
tisagenlecleucel-t (Kymriah™)	Novartis	DLBCL (relapsed/ refractory in 1st relapse)	IV	Phase 3 – sBLA; Breakthrough Therapy; Orphan Drug	TBD
tonogenchoncel-L	Kolon Tissuegene	Osteoarthritis (knee)	Intraarticular	Phase 3 – BLA	TBD
tradipitant	Vanda	Atopic dermatitis; Gastroparesis; Pruritus	Oral	Phase 3 – NDA	TBD
tralokinumab	AstraZeneca	Atopic dermatitis	SC	Phase 3 – BLA	TBD
transcon PEG growth hormone	Ascendis	Growth hormone deficiency	SC	Phase 3 – BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	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
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 (HBV) prevention	IM	Phase 3 – BLA	TBD
trofinetide	Acadia	Rett Syndrome	Oral	Phase 3 – NDA; Fast Track; Orphan Drug	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; Giant cell arteritis	Oral	Phase 3 – sNDA; Breakthrough Therapy	TBD
ustekinumab (Stelara)	Janssen	SLE	IV, SC	Phase 3 – sBLA	TBD
vilanterol trifenatate	GlaxoSmithKline	Asthma; COPD	Inhaled	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	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
vonoprazan	Phathom	*H. pylori* infection	Oral	Phase 3 – NDA; QIDP	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	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

Complete Response Letter (CRL)/Withdrawn Drugs

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
cabotegravir + rilpivirine (long-acting)	Viiv	HIV-1 infection	IM	CRL	TBD
naloxone single-dose prefilled syringe	Adamis	Substance use disorder	IM	CRL	TBD
oxycodegol	Nektar	Chronic low back pain	Oral	Withdrawal	N/A
RVT-802 (postnatal thymus tissue transplant)	Enzyvant	Pediatric congenital athymia	TBD	CRL	TBD
testosterone undecanoate	Lipocine	Hypogonadism	Oral	CRL	TBD
vedolizumab (Entyvio®)	Takeda	UC	IV, SC	CRL	TBD
vernakalant	Correvio	Atrial fibrillation	IV	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

ADL Activities of Daily Living

AED Anti-Epileptic Drug

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

ASCVD Atherosclerotic Cardiovascular Disease

AST Aspartate Aminotransferase

BLA Biologics License Application

BsUFA Biosimilar User Fee Act

BCVA Best Corrected Visual Acuity

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

DMD Duchenne Muscular Dystrophy

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-1RA Glucagon-Like Peptide-1 Receptor Agonist

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

MAOI Monoamine Oxidase Inhibitor

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 Physician 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

RSV Respiratory Syncytial Virus

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 Co-Transporter

SL Sublingual

SLE Systemic Lupus Erythematosus

SLL Small Lymphocytic Lymphoma

sNDA supplemental New Drug Application

SOC Standard of Care

sPGA Static Physician 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

TNBC Triple Negative Breast Cancer

TNFα Tumor Necrosis Factor-alpha

UA Unstable Angina

UC Ulcerative Colitis

US United States

UTI Urinary Tract Infection

VAS Visual Analog Scale

VEGF Vascular Endothelial Growth Factor

WBC White Blood Cell

WHO World Health Organization

XR Extended-Release

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