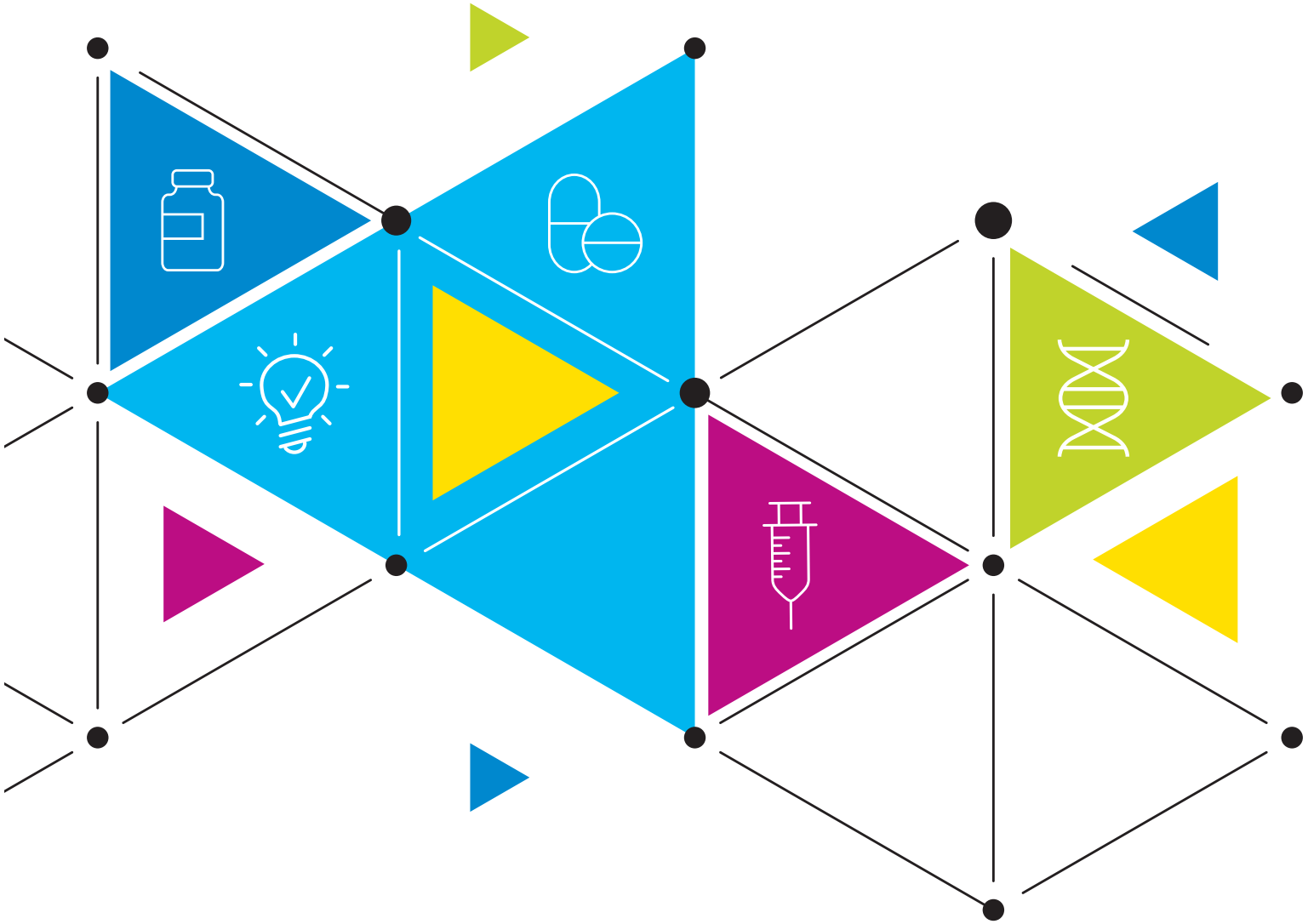
APRIL 2019

MRx Pipeline

A view into upcoming specialty and traditional drugs



Magellan Rx MANAGEMENT℠

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INTRODUCTION

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

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

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

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

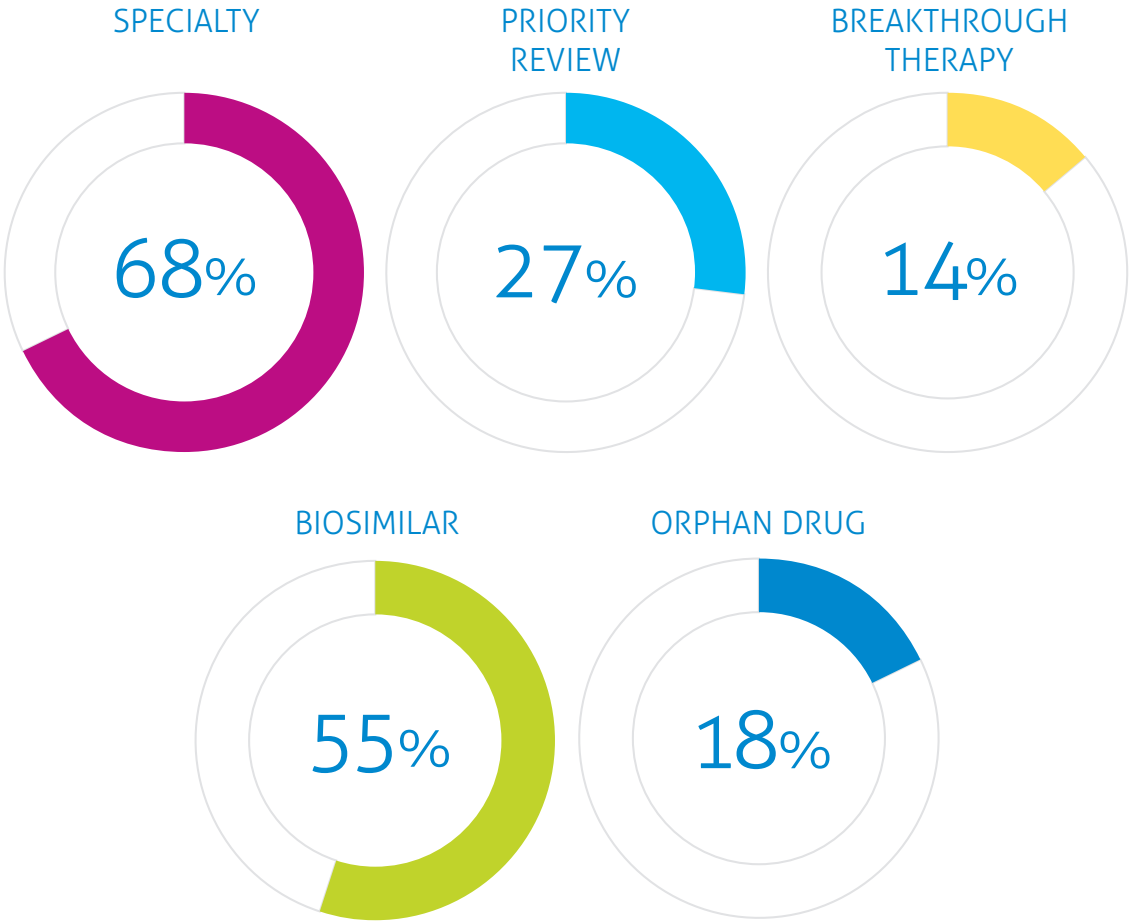
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year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

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

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

Pipeline Deep Dive

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



★ Specialty drug names appear in magenta throughout the publication.

entrectinib *oral*

Genentech

PROPOSED INDICATIONS

» Metastatic, ROS proto-oncogene 1 (ROS1)-positive non-small cell lung cancer (NSCLC)
» Neurotrophic tropomyosin receptor kinase (NTRK) fusion-positive, locally advanced or metastatic solid tumors

CLINICAL OVERVIEW

Entrectinib is an oral selective tyrosine kinase inhibitor (TKI) of the tropomyosin receptor kinases (TrkA, TrkB, TrkC), ROS1, and anaplastic lymphoma kinase (ALK) proteins. Typically, these oncogenes control the signaling of cellular growth, but when mutations occur, it can result in uncontrolled cell proliferation. Entrectinib binds to and inhibits TrkA, TrkB, TrkC, ROS1, and ALK, thereby leading to an inhibition of tumor cell proliferation and/or cell death in tumors that express these kinases.

Entrectinib was evaluated in one phase 2 trial (STARTRK-2) and two phase 1, dose escalation trials (STARTRK-1 and ALKA-372-001) that included patients with locally advanced or metastatic solid tumors displaying an NTRK1/2/3 (n=54), ROS1 (n=53), or ALK gene rearrangement. Cancer types included NSCLC, breast, cholangiocarcinoma, colorectal, gynecological, neuroendocrine, salivary gland, pancreatic, sarcoma, and thyroid.

Pooled data revealed responses across all tumor types. In patients with NTRK-positive solid tumors, after 15.5 months of follow-up, the overall objective response rate (ORR) was 57.4%, with a complete response rate of 7.4%. Median duration of response (DOR) was 10.4 months. In patients with intracranial involvement, ORR was 54.5%. In patients with ROS1-positive NSCLC, ORR was 77.4% and DOR was 24.6 months. Most adverse events were of grade 1 or 2 severity and were managed with dose adjustment or interruption. Anemia was the most frequently reported serious (grade 3 or 4) adverse event reported (11.8%), followed by weight gain (10.3%), and fatigue (7.4%). The ongoing STARTK-NG trial is evaluating entrectinib in pediatric patients with solid tumors.

PLACE IN THERAPY

NSCLC accounts for about 80% of all lung cancers and most patients present with advanced or metastatic disease upon diagnosis, of which about 5% are ALK-positive and 1% to 2% have ROS1 gene rearrangement. While, NTRK gene fusions are rare, they are not specific to any solid tumor location. Notably, an FDA-approved test to detect NTRK gene fusion is not currently available.

Several biomarkers (tumor mutations), including ROS1 and NTRK, have been identified and can be used in targeted therapy to improve patient response. If approved, entrectinib will most likely compete with crizotinib (Xalkori®), which is indicated to treat both ALK-positive and ROS1-positive NSCLC; entrectinib may be preferred over crizotinib in patients with CNS metastatic disease due to crizotinib's relatively poor CNS penetration. Alectinib (Alecensa®), ceritinib (Zykadia™), and lorlatinib (Lorbrena®) also have good CNS penetration and are FDA-approved for ALK-positive NSCLC.

Most solid tumors are treated based on tumor site rather than detected biomarkers. Entrectinib could be the second "tissue agnostic" kinase inhibitor, following the recently approved larotrectinib (Vitrakvi®), indicated to treat NTRK gene fusion positive solid tumors. Larotrectinib demonstrated a 75% ORR in this space and a similar incidence of grade 3/4 anemia (10%).

FDA APPROVAL TIMELINE

August 18, 2019

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$5	$55	$135	$195	$226

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

Musculoskeletal

golodirsen *IV*

Sarepta

PROPOSED INDICATIONS

Treatment of Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping

CLINICAL OVERVIEW

Golodirsen is an antisense oligonucleotide that binds to mutated exon 53 genetic code allowing the abnormal code to be "skipped" during the body's dystrophin manufacturing process, creating a partially functional dystrophin protein.

In a phase 1/2 trial that included males 7 to 13 years of age with DMD amenable to exon 53 skipping, all patients (n=25) exhibited a statistically significant increase in dystrophin protein in bicep muscle after 48 weeks of therapy; as measured by Western blot, a 10.7-fold increase in mean dystophin level was demonstrated (baseline, 0.095% of normal; on-treatment, 1.019% of normal). Increased intensity of dystrophin was also demonstrated. The phase 3 ESSENCE trial is ongoing and includes a blinded period in which male patients receive golodirsen or placebo for up to 96 weeks, followed by a 48-week extension phase during which all patients receive golodirsen. The primary efficacy measure is change in 6-minute walk test (6MWT) and secondary measures include maximum inspiratory and expiratory pressures. In the United Kingdom, the study was temporary halted due to 1 serious adverse event, possibly related to golodirsen. Estimated primary completion date of ESSENCE is in May 2022.

Golodirsen is being studied at 30 mg/kg as a once weekly IV infusion.

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 age 12. Moreover, death due to respiratory or cardiac failure typically occurs before age 30. Standard of care for DMD includes use of corticosteroids (prednisone, deflazacort [Emflaza™]) to delay progression of muscle weakness and improve respiratory function, but they are associated with side effects such as weight gain, bone fractures, and cataracts.

Golodirsen targets the exon 53 mutation and allows sections of defective genetic code to be bypassed during the dystrophin manufacturing process, creating a shorter partially functional dystrophin protein. Sarepta's eteplirsen (Exondys 51®), which targets exon 51, was the first antisense oligonucleotide approved for DMD. Sarepta has requested an Accelerated Approval process for golodirsen. Results from the ESSENCE trial will reveal if the modest increase (as viewed by industry experts) of dystrophin in muscle tissue will convey a meaningful clinical benefit. Notably, a similar increase in dystrophin was demonstrated with eteplirsen prior to its controversial FDA approval in 2016. The ESSENCE trial is also evaluating Sarepta's antisense oligonucleotide casimersen that targets exon 45 mutations; application for approval of casimersen is expected to be submitted to the FDA by mid-2019.

FDA APPROVAL TIMELINE

August 19, 2019

✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$25	$93	$176	$222	$257

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

lefamulin *IV, oral*

Nabriva

PROPOSED INDICATIONS

Community acquired nacterial pneumonia (CABP)

CLINICAL OVERVIEW

Lefamulin is a first-in-class, semi-synthetic pleuromutilin antibiotic with activity against gram-positive and atypical organisms associated with CABP.

In the phase 3, double-blind, LEAP-1 and LEAP-2 trials, lefamulin demonstrated non-inferiority to moxifloxacin in adults with CABP. In LEAP-1, a total of 551 patients were randomized (1:1) to IV lefamulin or IV moxifloxacin for 7 days; if MRSA was identified, then therapy was extended to 10 days in both groups and IV linezolid was added to moxifloxacin. Among available microbiological data, *Streptococcus pneumoniae* was reported in 59.7% of cases and *Haemophilus influenzae* in 34%. After 3 days, patients who were stable and afebrile could switch to oral therapy. Non-inferiority (12.5% margin) of lefamulin versus moxifloxacin ± linezolid was demonstrated based on early clinical response (ECR) 72 to 120 hours after the first dose (ECR, 87.3% versus 90.2%, respectively). Safety and tolerability were similar in both groups. In LEAP-2, a total of 738 patients were randomized (1:1) to receive 5 days of oral lefamulin or 7 days of oral moxifloxacin. ECR was 90.8% in each group and non-inferiority was met (10% margin). High ECR rates were reported across various pathogens in both groups, including 100% against MRSA. Notable differences in ECR occurred among penicillin-susceptible *S. pneumoniae* (lefamulin ECR, 76%; moxifloxacin ECR, 94.7%), *Moraxella catarrhalis* (85.7%, 100%), and *Legionella pneumophila* (81.3%, 94.1%). Diarrhea was reported more often with lefamulin compared to moxifloxacin (12.2% versus 1.1%); all cases were mild to moderate in severity and were of short duration (~2 days).

Study doses of lefamulin were 150 mg IV or 600 mg orally given every 12 hours. Oral and IV doses of moxifloxacin were given as 400 mg every 24 hours; in LEAP-1, oral and IV doses of linezolid were 600 mg given every 12 hours.

PLACE IN THERAPY

According to the CDC, about 1 million people seek care in a hospital due to pneumonia each year in the US. The most common etiology of CABP is *S. pneumoniae;* other causes include *H. influenzae, M.catarrhalis,* and *Staphylococcus aureus.* Empiric outpatient therapy includes a macrolide (with or without a beta-lactam), doxycycline, or a respiratory fluoroquinolone (moxifloxacin, gemifloxacin, levofloxacin). For MRSA pneumonia, IV vancomycin or oral or IV linezolid is recommended. Notably, available data report bacterial resistance to at least 1 antibiotic in 30% of pneumonia cases.

Lefamulin shows potent *in vitro* activity against CABP-associated pathogens, including *S. pneumoniae, H. influenzae, M. catarrhalis, S. aureus, Mycoplasma pneumoniae, Chlamydophila pneumoniae,* and *L. pneumophila.* It is also effective against organisms with resistance to major antibiotic classes. If approved, lefamulin will provide an important monotherapy option for adults with CABP. It displays a novel mechanism of action, including efficacy against treatment-resistant strains. Lefamulin could be an effective 5-day treatment alternative to fluoroquinolones, a class with numerous associated serious risks (tendon rupture, serious hypoglycemia, mental status changes, aortic ruptures). Lefamulin is also in phase 2 trials for the treatment of bacterial skin and skin structure infections.

FDA APPROVAL TIMELINE

August 19, 2019

✓ Fast Track ✓ Priority Review ✓ Qualified Infectious Disease Product

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$10	$66	$147	$230	$324

The forecast is a projection of total US sales per year for the lefamulin oral formulation only.

lumateperone *oral*

Intra-Cellular Therapies

PROPOSED INDICATIONS

Schizophrenia

CLINICAL OVERVIEW

Lumateperone is an atypical antipsychotic with activity as a serotonin-2A (5-HT2A) antagonist, dopaminergic and glutamatergic modulator, and serotonin reuptake inhibitor.

Efficacy of oral lumateperone was evaluated in one phase 2 trial (ITI-007-005) and two phase 3 trials (ITI-007-301, ITI-007-302). All were double-blind trials performed in adults (n=1,481 total) experiencing acute exacerbation of psychosis. While the magnitude of change in the Positive and Negative Syndrome Scale (PANSS) from baseline with lumateperone 60 mg daily was similar across all 3 trials (PANSS -13.4, -14.5, -14.6, respectively), the difference compared to placebo was not statistically significant in 1 of the trials ('302); the investigators suggested this was due to an unusually high placebo response (-15.1) at certain trial sites (placebo PANSS -7.4, -10.3, -15.1, respectively). Risperidone 4 mg was used as a control in 2 of the studies, both of which reported a statistically significant improvement in PANSS compared to placebo (trial '005: -13.4 versus -7.4, respectively; trial '302: -20.5 versus -15.1, respectively). Other lumateperone doses studied (20 mg, 40 mg, or 120 mg daily) did not show a significant improvement in PANSS compared to placebo. In all trials, the safety profile for lumateperone was similar to placebo. Furthermore, in a phase 3, open-label, long-term safety study (trial '303), stable patients with schizophrenia (n=603) were switched from standard of care (SOC) antipsychotics to lumateperone 60 mg. Use of lumateperone for up to 1 year resulted in significant improvements in body weight (mean change, -3.16 kg), stable blood glucose and insulin, reductions in lipid measures and prolactin levels, and little/no motor side effects. This benefit was lost in patients who were switched back to SOC.

PLACE IN THERAPY

Schizophrenia is an incurable devastating disorder characterized by disruptions in thought processes, perceptions, emotional responsiveness, and social interactions. Between 25% and 50% of schizophrenic patients attempt suicide, and 10% of patients succeed in their attempt. Schizophrenia is typically diagnosed in the late teen years to early thirties, and is more often seen in males than females. It is estimated that 2.2 million people in the US are affected by schizophrenia. The course of the illness varies among individuals ranging from little fluctuation in symptoms over time, to psychotic episodes lasting weeks or months.

By targeting the serotonergic and dopaminergic systems, lumateperone appears to reduce agitation and minimize positive schizophrenia symptoms (e.g., hallucination, delusion, racing thoughts). Its actions on glutamatergic signaling may help negative (e.g., apathy, poor social functioning) and cognitive symptoms of schizophrenia. If approved, lumateperone will provide an alternative to currently available atypical antipsychotic mediations used to treat schizophrenia. While lumateperone demonstrated a significant improvement in schizophrenia symptoms, the difference compared to placebo was less than the difference between risperidone (control) and placebo; however, lumateperone may find a place in treating patients with schizophrenia (acute episodes and stable symptoms) due to its favorable safety profile, particularly concerning cardiometabolic, endocrine, and motor parameters.

FDA APPROVAL TIMELINE

September 27, 2019

✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$22	$97	$179	$282	$384

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

Oncology

pexidartinib *oral*

Daiichi Sankyo

PROPOSED INDICATIONS

Tenosynovial giant cell tumor (TGCT) not amenable to improvement with surgery

CLINICAL OVERVIEW

TGCT is a rare condition characterized by benign tumors of the synovial lining of the joints, tendons sheaths, or bursae. Pexidartinib inhibits colony stimulating factor-1 receptor (CSF-1R), stem cell factor receptor (KIT), and FMS-like tyrosine kinase 3 (FLT3), leading to inhibition of tumor cell proliferation and down-regulation of macrophages, microglia, osteoclasts, and mast cells.

In the phase 3 ENLIVEN trial, 120 adults with symptomatic TGCT not amenable to surgery were randomized (1:1) to pexidartinib or placebo in a double-blind manner. After 24 weeks of treatment, the overall response rate (ORR) was 39% based on magnetic resonance imaging (MRI) (15% complete response) and 56% based on tumor volume score (TVS) in patients treated with pexidartinib compared to no response for either measure with placebo. Clinically significant improvement was demonstrated with pexidartinib compared to placebo regarding range of motion, physical function, and worst stiffness; however, a nonsignificant improvement in pain was experienced (31% versus 15%, respectively). Unlike placebo, hepatotoxicity was reported with pexidartinib (rate of AST or ALT ≥ 3 x upper limit of normal [ULN] was 33% and total bilirubin ≥ 2 x ULN was 5%). Onset of 4 serious cases occurred within the first 2 months of therapy; 8 patients discontinued therapy due to hepatic effects. Furthermore, in studies not related to TGCT treatment, pexidartinib-related toxicity resulted in liver transplant in 1 case and death in another. Patients who completed the blinded phase of the trial were eligible to continue to an ongoing open-label extension phase; to date, open-label data have not been published.

Pexidartinib was studied as an oral dose of 1,000 mg/day for 2 weeks followed by 800 mg/day thereafter.

PLACE IN THERAPY

TGCT, also known as pigmented villonodular synovitis (PVNS) or giant cell tumor of the tendon sheath (GCT-TS), is a potentially debilitating nonmalignant tumor of the joint or tendon sheath. Onset of TGST lesions usually occur between the ages of 25 and 40 years. Incidence is estimated to be 1.8 cases per 1 million people in the US, with a slightly higher incidence in women. TGCT lesions are classified as: localized (GCT), which occur most often in the hands and feet; or diffuse (PVNS), which can be more aggressive and destructive and affect larger joints (hips, knees). Current treatment is surgical excision; however, lesions often recur, and patients continue to experience declining physical function and QOL.

Pexidartinib has demonstrated significant response in adults with TGCT after 6 months of treatment; a long-term study is ongoing. While serious hepatic toxicity has been reported and may impact patient and prescriber willingness to use the product, if approved, pexidartinib will be the first systemic treatment for TGCT when surgery is not an option.

FDA APPROVAL TIMELINE

August 3, 2019

✓ Breakthrough Therapy ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$18	$35	$55	$67	$79

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

Neurology

tafamidis meglumine/tafamidis free acid *oral*

Pfizer

PROPOSED INDICATIONS

transthyretin amyloid cardiomyopathy (ATTR-CM)

CLINICAL OVERVIEW

Transthyretin (TTR) amyloid cardiomyopathy is a progressive, fatal disease caused by a buildup of amyloid fibrils in the heart. Amyloid fibrils collect when the transport protein transthyretin becomes unstable and misfolds. Tafamidis is an oral agent that stabilizes TTR, preventing amyloid fibrils from forming.

In the 30-month, double-blind ATTR-ACT trial (n=441), tafamidis meglumine was associated with a 30% reduction in all-cause mortality (29.5% versus 42.9%, respectively; p=0.0259) and 32% reduction in annualized rate of CV-related hospitalizations (0.48 versus 0.7, respectively; p<0.0001) compared to placebo. A difference between tafamidis meglumine and placebo was seen as early as 18 months of starting therapy. While degree of reduction in all-cause mortality was similar with hereditary and wild-type forms, a greater reduction was demonstrated in patients with less severe heart failure (New York Heart Association [NYHA] class I: 64.4%; class II: 39.6%; class III: 16.3%). Patients treated with tafamidis meglumine also experienced less decline in the 6MWT and certain QOL measures as early as 6 months after starting therapy. The safety profiles were similar for tafamidis meglumine and placebo.

Significant improvement was seen with daily oral doses of tafamidis meglumine 20 mg and 80 mg.

PLACE IN THERAPY

The incidence of ATTR varies widely by geographic region and ethnic group. Prevalence is estimated as 1 in 100,000 Americans of European descent. There are 2 types of ATTR-CM, a hereditary form due to mutated TTR protein (ATTRm) and a non-hereditary, wild-type form (ATTRwt). ATTR-CM is most often diagnosed in elderly men. Median survival after diagnosis is about 2.5 years for ATTRm and 3.6 years for ATTRwt. Presenting signs and symptoms in patients with ATTR-CM are fairly nonspecific and are often attributed to more common diseases affecting the heart (e.g., heart failure, diastolic dysfunction, arrhythmias). Moreover, some patients who inherit a TTR gene mutation may never develop symptoms; therefore, genetic testing of healthy individuals cannot predict whether a person will develop the condition.

Currently, there are no FDA-approved pharmacological options to treat ATTR-CM. Two agents, inotersen (Tegsedi™) and patisiran (Onpattro™), were approved in 2018 to treat polyneuropathy of hereditary transthyretin-mediated amyloidosis, but neither are indicated for ATTR-CM. Furthermore, standard treatments for CV disease such as beta-blockers, angiotensin-converting enzyme (ACE) inhibitors, angiotensin receptor blockers (ARBs), diuretics, or digoxin could worsen symptoms of ATTR-CM. Liver transplantation removes the source of variant TTR; however, accumulation of wild-type TTR can still occur. Off-label use of the oral NSAID diflunisal could stabilize TTR. Tafamidis is currently available in Europe and Japan. Pfizer has submitted applications to the FDA for 2 oral formulations of tafamidis: tafamidis meglumine 20 mg and tafamidis free acid 80 mg. While both formulations provide similar efficacy, tafamidis acid free is a more convenient option in patients who require an 80 mg dose of tafamidis. If approved, they will be the first agents in the US specifically indicated to treat ATTR-CM.

FDA APPROVAL TIMELINE

Tafamidis meglumine – July 2019

Tafamidis free acid – November 2019

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

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$23	$156	$392	$738	$1,139

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

Immunology

tenapanor *oral*

Ardelyx

PROPOSED INDICATIONS

Irritable bowel syndrome with predominantly constipation (IBS-C)

CLINICAL OVERVIEW

Tenapanor is a locally-acting, minimally absorbed sodium-hydrogen exchanger 3 (NHE3) inhibitor. It decreases sodium absorption from the GI tract resulting in increased intestinal fluid and improved intestinal transit.

The T3MPO study program evaluated the safety and efficacy of tenapanor in 1,203 adults (men and women) with IBS-C. In the T3MPO-1 and T3MPO-2 trials, response was defined as a combination of ≥ 30% reduction in abdominal pain and ≥ 1 complete spontaneous bowel movement (CSBM) in the same week for ≥ 6 of the 12 treatment weeks. Significantly more patients responded to therapy with tenapanor than with placebo (T3MPO-1: 27% versus 18.7%; T3MPO-2: 36.5% versus 23.7%, respectively). In both trials, tenapanor was also associated with statistically significant improvements in the secondary endpoints of abdominal pain and CSBM in ≥ 6 or ≥ 9 weeks of the 12 treatment weeks. The long-term extension T3MPO-3 trial (n=240) reported no safety concerns regarding serum chemistry or cardiac rhythm. Diarrhea was the most commonly reported adverse effect and led to discontinuation of tenapanor in 5.9% and 5.8% of patients in T3MPO-1 and T3MPR-2 trials, respectively, and 1.7% in T3MPO-3.

The trials evaluated a dose of tenapanor 50 mg administered orally twice daily.

PLACE IN THERAPY

IBS is a functional bowel disorder characterized by recurrent abdominal pain and abnormal bowel patterns. The life-long illness is typically diagnosed in relatively young individuals. IBS occurs in up to 15% of the population and is up to 2.5 times more common in women than men. More often, IBS is constipation predominant (IBS-C). Symptoms may improve with diet and/or the use of soluble fiber. FDA-approved products for IBS-C include linaclotide (Linzess®), lubiprostone (Amitiza®; for women only), and plecanatide (Trulance®). These work, at least in part, by increasing chloride concentration in the GI tract. Recently, tegaserod (Zelnorm®) was approved to return to the US market to treat IBS-C in women < 65 years of age; this followed a voluntary withdrawal of the product in 2007 due to a potential risk of CV ischemic events. Tegaserod is a selective serotonin-4 (5-HT4) receptor agonist that targets neurons and smooth muscle cells in the GI tract.

If approved, tenapanor will be the first NHE3 inhibitor in the US to treat IBS-C. Its most commonly reported side effect, diarrhea, is also an inherent adverse effect of the other 4 products indicated for IBS-C.

FDA APPROVAL TIMELINE

September 13, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$25	$81	$133	$187	$241

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

ubrogepant *oral*

Allergan

PROPOSED INDICATIONS

Acute treatment of migraine in adults

CLINICAL OVERVIEW

Ubrogepant is an oxazolidinone-based calcitonin gene-related peptide (CGRP) receptor antagonist.

Safety and efficacy of 3 doses of ubrogepant were evaluated for the treatment of acute migraine in the phase 3, double-blind, ACHIEVE-1 (50 mg and 100 mg) and ACHIEVE-2 (25 mg and 50 mg) trials. Over 3,300 adults, predominantly female, with a history of 2 to 8 moderate to severe migraine attacks (with or without aura) per month were enrolled to treat a single migraine attack. In both studies, patients had the option to take a second oral dose (placebo-matching ubrogepant or rescue medication) 2 to 48 hours after the initial dose.

In both trials, compared to placebo, all doses of ubrogepant were associated with a significantly greater proportion of patients reported to be free of migraine pain 2 hours after the initial dose; in ACHIEVE-1, response rates were: 19.2%, 21.2%, and 11.8% for ubrogepant 50 mg and 100 mg, and placebo, respectively; in ACHIEVE-2, response rates were: 20.7%, 21.8%, and 14.3% for ubrogepant 25 mg and 50 mg, and placebo, respectively. Two hours after the initial dose, absence of the most bothersome symptom (MBS), including photophobia, phonophobia, or nausea, was also significantly better with ubrogepant than placebo; in ACHIEVE-1, absence of MBS was reported in 38.6%, 37.7%, and 27.8% of patients treated with ubrogepant 50 mg and 100 mg, and placebo, respectively; in ACHIEVE-2, absence of MBS was reported in 38.9% and 27.4% of patients treated with ubrogepant 50 mg and placebo, respectively, and the difference with ubrogepant 25 mg was not statistically significant. Adverse events with ubrogepant were comparable to placebo. Across all treatment arms in both trials, 10 cases of elevated liver function test values > 3 times the ULN were reported; however, none were considered related to study drug. Furthermore, no new safety concerns, including elevated transaminases, were identified with ubrogepant treatment (50 mg and 100 mg) in the 52-week, open-label extension study (n=1,254).

PLACE IN THERAPY

Over 37 million Americans, mostly women, suffer from migraine headaches. Attacks can be debilitating, with pain lasting hours to days. Triptans are the current standard of care to treat acute, moderate to severe migraine episodes. In 2018, the FDA approved 3 injectable monoclonal antibodies that target the CGRP receptor (erenumab [Aimovig™], fremanezumab [Ajovy™], and galcanezumab [Emgality™]) for migraine prophylaxis. If approved, ubrogepant tablets will be the first oral CGRP receptor antagonist for acute treatment and will likely compete with triptans and/or the oral investigational CRGP-targeted rimegepant (tablet and ODT formulations) expected for FDA submission in Q2, 2019. While the development of 2 other CGRP antagonists were suspended due to liver toxicity, ubrogepant and rimegepant have not been determined to cause increased liver enzymes.

Other agents that target CGRP in the pipeline include the oral CRGP receptor antagonist atogepant (dosed once or twice daily) which is in late-phase trials for migraine prophylaxis. Regarding injectable monoclonal antibody CGRP inhibitors, galcanezumab was submitted to the FDA for the preventive treatment of episodic cluster headache (approval is expected in June 2019) and investigational IV eptinezumab was submitted for migraine prophylaxis (approval is expected in February 2020).

FDA APPROVAL TIMELINE

December 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$0	$27	$104	$189	$273

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

upadacitinib *oral*

Abbvie

PROPOSED INDICATIONS

Rheumatoid arthritis (RA)

CLINICAL OVERVIEW

Upadacitinib is a selective Janus kinase (JAK)-1 inhibitor.

The pivotal SELECT program consisted of 5 phase 3 trials that evaluated daily upadacitinib in adults with moderate to severe RA. In the completed 12-week SELECT-BEYOND and SELECT-NEXT trials, upadacitinib was superior to placebo when given in combination with background conventional synthetic DMARDs (csDMARDs). Interim data from SELECT-COMPARE revealed upadacitinib 15 mg was superior to adalimumab (40 mg every other week) when each were given with methotrexate (ACR50, 45.5% versus 29.1%, respectively; DAS28CRP ≤ 3.2, 45% versus 28.7%, respectively). The SELECT-EARLY and SELECT-MONOTHERAPY trials showed upadacitinib, as monotherapy or in combination with methotrexate, was superior to methotrexate alone; respective response rates in each trial were: SELECT-EARLY–ACR50, 52.1%-56.4% versus 28.3% and DAS28CRP < 2.6, 48.3%-50% versus 18.5%; SELECT-MONOTHERAPY–ACR20, 67.7%-71.2% versus 41.2% and DAS28CRP ≤ 3.2, 44.7%-53% versus 19.4%). Across the RA studies, no safety signals were detected for upadactinib, including risk of thromboembolic events.

Upadacitinib was studied as daily oral doses of 15 mg and 30 mg.

PLACE IN THERAPY

An estimated 1.3 million adults in the US suffer from RA. First-line treatment of RA is use of DMARDs (methotrexate preferred). If disease activity remains moderate or severe, use of combination DMARDs, an anti-tumor necrosis factor (TNF) agent, a non-TNF biologic, or the JAK inhibitor tofacitinib (all with or without methotrexate) is recommended. Anti-TNF agents and non-TNF biologics are preferred over tofacitinib (Xeljanz®/Xeljanz XR®).

Intracellular Janus kinase (JAK) proteins are activated in response to immune stimulus and lead to inflammation and tissue destruction. Currently available oral JAK inhibitors, both with once daily dosing, are baricitinib (Olumiant™; JAK 1 and JAK2 inhibitor) and tofacitinib (Xeljanz/Xeljanz XR; JAK1 and JAK3 inhibitor). Tofacitinib is indicated after inadequate response or intolerance to methotrexate and can be used as monotherapy or in combination with methotrexate or other nonbiologic DMARDs. Baricitinib is indicated in patients with an inadequate response to ≥ 1 TNF inhibitor. Upadacitinib was studied in patients with an inadequate response to a csDMARD or biologic DMARD and in those who were methotrexate-naïve.

If approved, upadacitinib's apparent greater selectivity for JAK1 could provide a better efficacy and safety profile compared to other JAK inhibitors, including lack of dose-limiting anemia that is reported with tofacitinib and baricitinib. Moreover, both tofacitinib and baricitinib are associated with an increased risk of thromboembolic events, which has not been seen with upadacitinib in clinical trials. Prescriber experience with tofacitinib and increasing availability of anti-TNF biosimilars may limit upadacitinib's market uptake. Tofacitinib is also approved for psoriatic arthritis and ulcerative colitis, indications that upadacitinib is in late-stage development along with Crohn's disease, atopic dermatitis, and axial spondyloarthritis.

FDA APPROVAL TIMELINE

August 20, 2019

✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$22	$209	$580	$854	$1,043

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

Biosimilar Overview

CLINICAL OVERVIEW

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

Many controversies surround biosimilars, but regulatory and litigation hurdles remain. The FDA has issued final and draft guidances. In 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 the pharmacists' ability to substitute an interchangeable biosimilar for the reference product. Although the agency has not released its final guidance on interchangeability, several states have already enacted biosimilar substitution legislation. The FDA withdrew the September 2017 draft industry guidance on determining similarity of a proposed biosimilar product to its reference product to allow for further consideration of the most current and relevant scientific methods in evaluating analytical data. The agency will focus on providing flexibility for efficient development of biosimilars while maintaining high scientific standards. In July 2018, the FDA finalized its guidance on labeling biosimilars. The guidance pertains to prescribing information (PI) but does not contain specific recommendations on interchangeability in the labeling. The labeling guidance provides recommendations on how to include, identify, and differentiate the biosimilar versus the reference product in various sections of the PI. The basic premise remains that the originator product's safety and effectiveness can be relied upon for HCPs to make prescribing decisions; therefore, a biosimilar should include relevant data from the originator in its PI.

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

Insulins are historically regulated by the FDA as small molecules. Since the reference products are not deemed biologics by the FDA, any generics are technically branded competitors and not considered biosimilars under the FDA's definition. In practice, however, *follow-on* insulins are regarded to be complex molecules and considered in the biosimilar space. In December 2018, the FDA announced its plans to transition a small subset of biologics currently approved as drugs under the Federal Food, Drug, and Cosmetics Act to be licensed as biologics. Starting in March 2020, drugs such as insulin and growth hormone will be deemed biologics and transition from the drug pathway to the biologics pathway. This new categorization can promote competition and access.

PLACE IN THERAPY

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

In July 2018, the FDA unveiled its Biosimilar Action Plan (BAP), a series of 11 steps to encourage biosimilar market competition, some of which were previously announced or underway. 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 19 biosimilars have received FDA approval. Of these, only 7 have entered the market.

APPROVED BIOSIMILARS				
Brand Name (Nonproprietary name)	**Manufacturer**	**Approval Date**	**Commercially Available**	**Originator Product (Manufacturer)**
Zarxio® (filgrastim-sndz)	Sandoz	March 2015	✓	Neupogen® (Amgen)
Inflectra (infliximab-dyyb)	Pfizer/ Celltrion	April 2016	✓	Remicade® (Janssen)
Erelzi™ (etanercept-szzs)	Sandoz	August 2016	-	Enbrel® (Amgen)
Amjevita™ (adalimumab-atto)	Amgen	September 2016	-	Humira® (Abbvie)
Renflexis (infliximab-abda)	Merck	May 2017	✓	Remicade (Janssen)
Cyltezo® (adalimumab-adbm)	Boehringer Ingelheim	August 2017	-	Humira (Abbvie)
Mvasi™ (bevacizumab-awwb)	Amgen	September 2017	-	Avastin® (Genentech)
Ixifi™ (infliximab-qbtx)*	Pfizer	December 2017	-	Remicade (Janssen)
Ogivri™ (trastuzumab-dkst)	Mylan	December 2017	-	Herceptin® (Genentech)
Retacrit™ (epoetin alfa-epbx)	Pfizer/ Hospira	May 2018	✓	Epogen® (Amgen) Procrit® (Janssen)
Fulphila™ (pegfilgrastim-jmdb)	Mylan	June 2018	✓	Neulasta® (Amgen)
Nivestym™ (filgrastim-aafi)	Pfizer	July 2018	✓	Neupogen (Amgen)
Hyrimoz™ (adalimumab-adaz)	Sandoz	October 2018	-	Humira (Abbvie)
Udenyca™ (pegfilgrastim-cbqv)	Coherus	November 2018	✓	Neulasta (Amgen)
Truxima (rituximab-abbs)	Celltrion/Teva	November 2018	-	Rituxan® (Genentech)
Herzuma® (trastuzumab-pkrb)	Celltrion/Teva	December 2018	-	Herceptin (Genentech)
Ontruzant® (trastuzumab-dttb)	Samsung Bioepis/ Merck	January 2019	-	Herceptin (Genentech)
Trazimera™ (trastuzumab-qyyp)	Pfizer	March 2019	-	Herceptin (Genentech)
Eticovo™ (etanercept-ykro)	Samsung Bioepis/ Merck	April 2019	-	Enbrel (Amgen)

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

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

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

While < 2% of Americans use biologics, they account for almost 40% of all prescription drug spending. Moreover, they comprised 70% of growth in drug spending from 2010 to 2015. Not surprisingly, there is a growing body of evidence on predicted biologic spend and potential biosimilar savings. The global biologic market is projected to exceed $390 billion by 2020. The global biosimilar market is expected to grow from $5.95 billion in 2018 to $23.63 billion in 2023. An IMS Health analysis expects biosimilars to save the US and Europe's top 5 markets up to $110

billion by 2020. It is estimated that in the US, biosimilars will cost 15% to 35% less than the originator product. The potential cost savings, however, can vary based on the market segment where brand contracts can play a role. A 2017 report by the RAND Corporation estimates a $54 billion cost savings from biosimilars between 2017 and 2026. In July 2018, an FDA analysis reported that if Americans had access to FDA-approved biosimilars in 2017, it would have resulted in a $4.5 billion savings. A 2017 analysis by the Moran Company projects biosimilars can save the government an estimated $11.4 billion by 2027, but it would require the CMS to revise its reimbursement policy for biosimilars. Subsequently, in November 2017, CMS revised its reimbursement policy. The CMS has begun to issue a unique Healthcare Common Procedure Coding System (HCPCS) code to each individual biosimilar. Under this new rule, Medicare Part B will separately code and pay for biosimilars and no longer group them into a common payment code with originator agents. A June 2018 study by the Pacific Research Institute forecasts annual savings of up to $465 million from increased use of biosimilars to replace a single biologic for commercial payers and Medicare, based on an infliximab case study.

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

Blood Modifier
adalimumab *SC*

PF-06410293 and SB5 are biosimilars to Abbvie's Humira, a tumor necrosis factor alpha (TNF-α) blocker indicated for the treatment of autoimmune disorders, including rheumatoid arthritis (RA), juvenile idiopathic arthritis (JIA), ankylosing spondylitis (AS), plaque psoriasis (PSO), psoriatic arthritis (PsA), Crohn's disease (CD) in adults and children, ulcerative colitis (UC), hidradenitis suppurativa (HS), and non-infectious uveitis.

FDA APPROVAL TIMELINE

Pfizer (PF-06410293)
Q4, 2019

Samsung Bioepis/Merck (SB5)
May 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$14,718	$15,676	$16,346	$16,788	$13,554

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

Oncology
bevacizumab (PF-06439535) *IV*

Pfizer

PF-06439535 is a biosimilar to Genentech's Avastin, a vascular endothelial growth (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

Q2, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$2,824	$2,338	$1,749	$1,318	$1,101

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

Blood Modifier
filgrastim *IV, SC*

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

FDA APPROVAL TIMELINE

Adello
Pending

Apotex (Grastofil)
Pending

Tanvex (TX01)
August 1, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$158	$136	$122	$111	$72

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

Immunology
infliximab (ABP-710) *IV*

Amgen

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

FDA APPROVAL TIMELINE

October 17, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$ 2,943	$2,395	$2,000	$1,666	$1,402

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

Blood Modifier
pegfilgrastim *SC*

Lapelga and LA-EP2006 are biosimilars to Amgen's Neulasta, a leukocyte growth factor indicated for use in patients with nonmyeloid malignancies receiving myelosuppressive anti-cancer drugs and patients acutely exposed to myelosuppressive doses of radiation (HSARS).

FDA APPROVAL TIMELINE

Apotex (Lapelga)
Pending

Novartis (LA-EP2006)
October 3, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$3,187	$2,677	$2,266	$1,934	$1,617

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

Oncology
rituximab (PF-05280586) *IV*

Pfizer

PF-05280586 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-associated vasculitis.

FDA APPROVAL TIMELINE

Q2, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$4,030	$3,385	$2,692	$1,972	$1,622

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

Endocrine
teriparatide (PF708) *SC*

Pfenex

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

FDA APPROVAL TIMELINE
October 7, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$601	$443	$345	$290	$244

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

Oncology
trastuzumab (Kanjinti) *IV*

Amgen/Allergan

Kanjinti is an investigational biosimilar to Genentech's Herceptin, a HER2/neu receptor antagonist indicated for the treatment of HER2-positive breast cancer and HER2-positive metastatic gastric or gastroesophageal junction adenocarcinoma.

FDA APPROVAL TIMELINE
June 2019

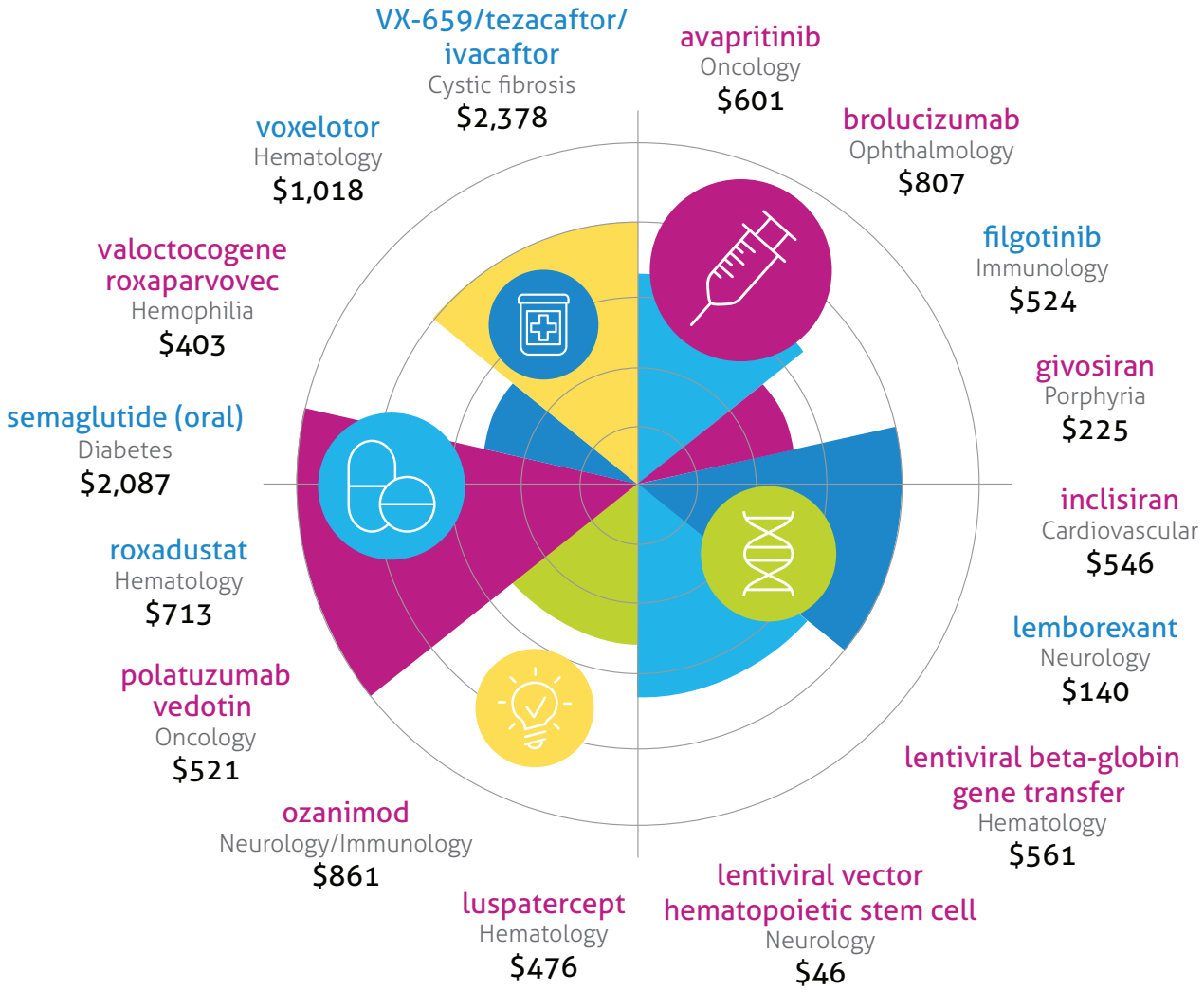
FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$2,793	$2,346	$1,932	$1,621	$1,296

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

Keep on Your Radar

Notable agents that are further from approval have been identified in this unique watch list. These are products with the potential for significant clinical and financial impact. Their development status is being tracked on the *MRx Pipeline* radar. These pipeline products, their respective class or proposed indication, as well as an estimated financial forecast for the year 2023, are displayed. The financials are projected total annual US sales, reported in *millions.*

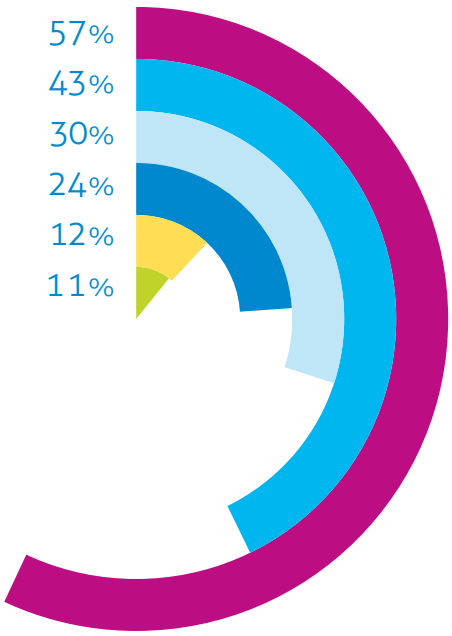


★ Specialty drug names appear in magenta throughout the publication.

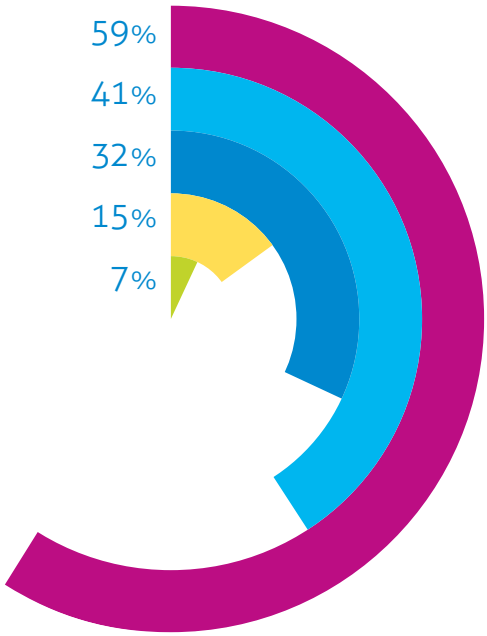
Pipeline Drug List

The pipeline drug list is an aerial outline of drugs with anticipated FDA approval through 2020. It is not intended to be a comprehensive inventory of all drugs in the pipeline; emphasis is placed on drugs in high-impact categories. Investigational drugs with a Complete Response Letter (CRL) and those that have been withdrawn from development are also noted.

APPLICATION SUBMITTED TO THE FDA



IN PHASE 3 TRIALS



Specialty | Traditional | Priority Review | Orphan Drug | Breakthrough Therapy | Biosimilar

★ Specialty drug names appear in magenta throughout the publication.

PIPELINE DRUG LIST

★ Specialty drug names appear in magenta throughout the publication.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
DS-300 (nutritional)	Eton	Nutritional deficiencies	SC	Submitted - 505(b)(2) NDA; Fast Track	2019
ramucirumab (Cyramza®)	Eli Lilly	HCC (2nd-line)	IV	Submitted - sBLA; Orphan Drug	Apr-May 2019
bevacizumab (biosimilar to Genentech's Avastin)	Pfizer	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Submitted - BLA	Apr-Jun 2019
rituximab (biosimilar to Genentech's Rituxan)	Pfizer	NHL; RA	IV	Submitted - BLA	Apr-Jun 2019
glucagon	Eli Lilly	Hyperinsulinemia/ hypoglycemia	Intranasal	Submitted - NDA	Apr-Jul 2019
bupivacaine/meloxicam	Heron	Postsurgical pain	Instillation	Submitted - NDA; Breakthrough Therapy; Fast Track; Priority Review	04/30/2019
adalimumab (biosimilar to Abbvie's Humira)	Samsung Bioepis/Merck	RA; AS; PSO; PsA; JIA; CD; UC	SC	Submitted - BLA	May 2019
dapagliflozin (Farxiga®)	AstraZeneca	T2DM with CDK	Oral	Submitted - sNDA	May 2019
exenatide ER (Bydureon®)	AstraZeneca	T2DM CV outcomes	SC	Submitted - sNDA	May 2019
onasemnogene abeparvovec	Novartis	Spinal muscular atrophy	IV	Submitted - BLA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	May 2019
cariprazine (Vraylar®)	Allergan	Bipolar depression	Oral	Submitted - sNDA	May-Jun 2019
dengue vaccine	Sanofi	Dengue fever	SC	Submitted - BLA; Fast Track; Priority Review	05/01/2019
amisulpride	Acacia	Post-operative nausea/ vomiting	IV	Submitted - NDA	05/05/2019
aflibercept (Eylea®)	Regeneron	Diabetic retinopathy (non-proliferative; regardless of diabetic macular edema)	Intraocular	Submitted - sBLA	05/13/2019
ruxolitinib (Jakafi®)	Incyte	GVHD	Oral	Submitted - sNDA; Breakthrough Therapy; Orphan Drug; Priority Review	05/24/2019
NKTR-181	Nektar	Chronic low back pain	Oral	Submitted - NDA; Fast Track	05/28/2019
avelumab (Bavencio®)	Merck	RCC (in combination with axitinib)	IV	Submitted - sBLA; Breakthrough Therapy; Priority Review	June 2019
galcanezumab-gnlm (Emgality®)	Eli Lilly	Cluster headache (episodic)	SC	Submitted - sBLA; Breakthrough Therapy; Fast Track; Priority Review	June 2019
trastuzumab (biosimilar to Genentech's Herceptin)	Amgen/Allergan	Breast cancer; Gastric/ gastroesophageal cancer	IV	Submitted - BLA	June 2019
onabotulinumtoxinA (Botox®)	Allergan	Upper limb spasticity (ages ≥ 2 years)	IM	Submitted - sBLA; Priority Review	Jun-Jul 2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ceftolozane/tazobactam (Zerbaxa®)	Merck	HAP	IV	Submitted - sNDA; Fast Track; Priority Review; QIDP	06/03/2019
pembrolizumab (Keytruda®)	Merck	SCCHN (recurrent, metastatic, monotherapy or in combination with chemotherapy)	IV	Submitted - sBLA; Priority Review	06/17/2019
mannitol	Pharmaxis	CF	Inhaled	Submitted - NDA; Fast Track; Orphan Drug	06/20/2019
bremelanotide	AMAG	Female sexual arousal disorder	SC	Submitted - NDA	06/21/2019
ivosidenib (Tibsovo®)	Agios	AML (1st-line, IDH1+, chemotherapy ineligible)	Oral	Submitted - sNDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	06/21/2019
celiprolol	Acer	Ehlers-Danlos syndrome (vascular)	Oral	Submitted - NDA; Orphan Drug; Priority Review	06/25/2019
dupilumab (Dupixent®)	Regeneron	Nasal polyposis	SC	Submitted - sBLA; Priority Review	06/26/2019
lenalidomide (Revlimid®)	Celgene	Follicular lymphoma (relapsed/refractory; in combination with rituximab); Marginal zone lymphoma (relapsed/ refractory; in combination with rituximab)	Oral	Submitted - sNDA; Orphan Drug; Priority Review	06/27/2019
eculizumab (Soliris®)	Alexion	Neuromyelitis optica (Devic's syndrome)	IV	Submitted - sBLA; Orphan Drug; Priority Review	06/28/2019
avatrombopag (Doptelet®)	Dova	Immune thrombocytopenia purpura (ITP)	Oral	Submitted - sNDA	06/30/2019
fosfomycin	Nabriva	UTI (complicated)	IV	Submitted - 505(b)(2) NDA; Fast Track; Priority Review; QIDP	06/30/2019
riluzole	Biohaven	Amyotrophic lateral sclerosis	SL	Submitted - 505(b)(2) NDA	July 2019
tafamidis meglumine	Pfizer	Transthyretin amyloid cardiomyopathy (ATTR-CM)	Oral	Submitted - NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	July 2019
pitolisant	Harmony	Narcolepsy	Oral	Submitted - NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	Jul-Aug 2019
selinexor	Karyopharm	Multiple myeloma (penta-refractory)	Oral	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	07/06/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
afamelanotide	Clinuvel	Erythropoietic porphyria	Intradermal	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	07/08/2019
relebactam/imipenem/cilastatin	Merck	Intra-abdominal infections (complicated); UTI (complicated)	IV	Submitted - NDA; Fast Track; Priority Review; QIDP	07/16/2019
apremilast (Otezla®)	Celgene	Behçet syndrome	Oral	Submitted - sNDA; Orphan Drug	07/19/2019
ferric maltol	Shield	Iron deficiency anemia	Oral	Submitted - NDA	07/26/2019
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis	SC	Submitted - sBLA	August 2019
pretomanid	Mylan	Tuberculosis (treatment resistant/nonresponsive)	Oral	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	Aug-Sep 2019
filgrastim (biosimilar to Amgen's Neupogen)	Tanvex	Neutropenia/leukopenia	SC	Submitted - BLA	08/01/2019
pexidartinib	Daiichi Sankyo	Tenosynovial giant cell tumor (TGCT)	Oral	Submitted - NDA; Breakthrough Therapy; Orphan Drug; Priority Review	08/03/2019
cefiderocol	Shionogi	UTI (complicated)	IV	Submitted NDA	08/14/2019
loteprednol etabonate 0.25%	Kala	Dry eye	Topical	Submitted - 505(b)(2) NDA	08/15/2019
tasimelteon (Hetlioz®)	Vanda	Jet lag disorder	Oral	Submitted - sNDA	08/16/2019
entrectinib	Genentech	Solid tumors (NTRK fusion-positive, locally advanced or metastatic); NSCLC (metastatic, ROS1-positive)	Oral	Submitted - NDA; Breakthrough Therapy; Orphan Drug; Priority Review	08/18/2019
golodirsen	Sarepta	Duchenne muscular dystrophy	IV	Submitted - NDA; Orphan Drug; Priority Review	08/19/2019
lefamulin	Nabriva	CABP	IV, Oral	Submitted - NDA; Fast Track; Priority Review; QIDP	08/19/2019
polatuzumab vedotin	Roche	DLBCL	IV	Submitted - BLA; Breakthrough Therapy; Orphan Drug; Priority Review	08/19/2019
upadacitinib	Abbvie	RA	Oral	Submitted - NDA; Priority Review	08/20/2019
quizartinib	Daiichi Sankyo	AML (FLT3-ITD mutated)	Oral	Submitted - NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	08/25/2019
istradefylline	Kyowa Hakko Kirin	Parkinson's disease (Off episodes)	Oral	Submitted - NDA	08/27/2019
oxycodone ER (abuse- and alcohol-resistant)	Intellipharmaceutics	Chronic pain	Oral	Submitted - 505(b)(2) NDA; Fast Track	08/28/2019
dapagliflozin (Farxiga)	AstraZeneca	T1DM	Oral	Submitted - sNDA	September 2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
smallpox vaccine	Bavarian Nordic	Smallpox	SC	Submitted - BLA; Priority Review	September 2019
atezolizumab (Tecentriq®)	Genentech	NSCLC (1st-line, metastatic non-squamous, EGFR-negative, ALK-negative, in combination with Abraxane and carboplatin)	IV	Submitted - sBLA	09/02/2019
fedratinib	Celgene	Myelofibrosis	Oral	Submitted - NDA; Priority Review	09/03/2019
obinutuzumab (Gazyva®)	Genentech	CLL/SLL (treatment-naïve; in combination with venetoclax)	IV	Submitted - sBLA; Breakthrough Therapy; Orphan Drug	09/06/2019
tenapanor	Ardelyx	IBS-C	Oral	Submitted - NDA	09/13/2019
nintedanib (Ofev®)	Boehringer Ingelheim	Scleroderma	Oral	Submitted - sNDA; Fast Track; Orphan Drug	09/18/2019
semaglutide (Ozempic®)	Novo Nordisk	T2DM	Oral	Submitted - NDA; Priority Review	09/20/2019
lumateperone	Intra-Cellular Therapies	Schizophrenia	Oral	Submitted - NDA; Fast Track	09/27/2019
cetirizine	Pfizer	Urticaria	IV	Submitted - 505(b)(2) NDA	Oct-Nov 2019
deflazacort (Emflaza)	PTC Therapeutics	Duchenne muscular dystrophy (ages 2-5 years)	Oral	Submitted - sNDA; Fast Track; Orphan Drug	Oct-Nov 2019
lorcaserin (Belviq®)	Eisai	Obesity	Oral	Submitted - sNDA	Oct-Nov 2019
lorcaserin ER (Belviq XR®)	Eisai	Obesity	Oral	Submitted - sNDA	Oct-Nov 2019
onabotulinumtoxinA (Botox)	Allergan	Lower limb spasticity (ages ≥ 2 years)	IM	Submitted - sBLA	Oct-Nov 2019
adalimumab (biosimilar to Abbvie's Humira)	Pfizer	RA; AS; PSO; PsA; JIA; CD; UC	SC	Submitted - BLA	Oct-Dec 2019
pegfilgrastim (biosimilar to Amgen's Neulasta)	Novartis	Neutropenia/leukopenia	SC	Submitted - BLA	10/03/2019
emtricitabine/tenofovir alafenamide (Descovy®)	Gilead	HIV-1 infection pre-exposure prophylaxis (PrEP)	Oral	Submitted - sNDA; Priority Review	10/04/2019
teriparatide recombinant, human (follow-on to Eli Lilly's Forteo)	Pfenex	Osteoporosis	SC	Submitted - 505(b)(2) NDA	10/07/2019
rivaroxaban (Xarelto®)	Janssen	VTE prevention in medically ill patients	Oral	Submitted - sNDA	10/14/2019
diroximel fumarate	Biogen	MS (relapsing)	Oral	Submitted - 505(b)(2) NDA	10/17/2019
infliximab (biosimilar to Janssen's Remicade)	Amgen	RA	IV	Submitted - BLA	10/17/2019
triamcinolone ER (Zilretta®)	Flexion	Osteoarthritis of the knee (repeat dosing)	Intra-articular	Submitted - sNDA	10/17/2019
minocycline	Foamix	Acne (ages ≥ 9 years)	Topical	Submitted - 505(b)(2) NDA	10/18/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
romiplostim (Nplate®)	Amgen	Immune thrombocytopenia purpura (ITP) (resistant)	SC	Submitted - sBLA	10/18/2019
ustekinumab (Stelara®)	Janssen	UC	IV, SC	Submitted - sBLA; Orphan Drug	10/18/2019
triamcinolone acetonide	Clearside Biomedical	Macular edema associated with uveitis	Intraocular	Submitted - 505(b)(2) NDA	10/19/2019
darolutamide	Bayer	Prostate cancer (non-metastatic, castration-resistant)	Oral	Submitted - NDA; Fast Track; Priority Review	10/25/2019
naloxone hydrochloride	Adamis	Substance use disorder	IM	Submitted - 505(b)(2) NDA	10/31/2019
brolucizumab	Novartis	Wet AMD	Intraocular	Submitted - BLA; Priority Review	November 2019
methotrexate	Cumberland	PSO	SC	Submitted - NDA	November 2019
tafamidis free acid	Pfizer	Transthyretin amyloid cardiomyopathy (ATTR-CM)	Oral	Submitted - NDA	November 2019
baloxavir marboxil (Xofluza®)	Genentech	Influenza vaccine (pediatrics)	Oral	Submitted - sNDA	11/4/2019
dexamethasone (Dextenza®)	Ocular Therapeutix	Ocular inflammation (post-surgical)	Intraocular	Submitted - sNDA	11/10/2019
lasmiditan	Eli Lilly	Migraine treatment (adults)	Oral	Submitted - NDA	11/14/2019
cenobamate	SK Biopharmaceuticals	Parital-onset seizure	Oral	Submitted - NDA	11/21/2019
riluzole film	Aquestive	Amyotrophic lateral sclerosis	Oral Transmucosal	Submitted - 505(b)(2) NDA; Orphan Drug	11/30/2019
ubrogepant	Allergan	Migraine treatment (adults)	Oral	Submitted - NDA	December 2019
lemborexant	Eisai	Insomnia	Oral	Submitted - NDA	12/27/2019
AR101	Aimmune	Peanut allergy	Oral	Submitted - BLA; Breakthrough Therapy; Fast Track	January 2020
icosapent ethyl (Vascepa®)	Amarin	Major CV event risk reduction	Oral	Submitted - sNDA	January 2020
insulin aspart (Fiasp®)	Novo Nordisk	T1DM (pediatrics)	SC	Submitted - sNDA	01/01/2020
daratumumab (Darzalex®)	Janssen	Multiple myeloma (1st-line; ineligible for SCT; in combination with lenalidomide and dexamethasone)	IV	Submitted - sBLA; Orphan Drug	01/12/2020
semaglutide (Ozempic)	Novo Nordisk	T2DM CV outcomes	SC	Submitted - sNDA	01/20/2020
daratumumab (Darzalex)	Janssen	Multiple myeloma (1st-line; eligible for SCT; in combination with bortezomib, thalidomide, and dexamethasone)	IV	Submitted - sBLA; Orphan Drug	01/24/2020
canagliflozin (Invokana®)	Janssen	Diabetic nephropathy risk reduction with T2DM	Oral	Submitted - sNDA	01/28/2020

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
risperidone ER (microsphere)	Luye	Schizophrenia; Bipolar disorder	IM	Submitted - 505(b)(2) NDA	01/28/2020
leuprolide mesylate depot (ready-to-use)	Foresee	Prostate cancer	SC	Submitted - 505(b)(2) NDA	01/29/2020
bempedoic acid	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted - NDA	02/20/2020
bempedoic acid/ezetimibe	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Submitted - NDA	02/20/2020
eptinezumab	Alder Bio	Migraine prevention	IV	Submitted - BLA	02/21/2020
apalutamide (Erleada®)	Janssen	Prostate cancer (metastatic, castration-sensitive)	IV	Submitted - sNDA	02/28/2020
cabotegravir	GlaxoSmithKline	HIV-1 infection	IM	Submitted - NDA	02/28/2020
rilpivirine (long-acting)	Janssen	HIV-1 infection	IM	Submitted - NDA	02/28/2020
trifarotene	Galderma	Acne	Topical	Submitted - NDA	03/01/2020
ozanimod	Celgene	MS (relapsing)	Oral	Submitted - NDA	03/25/2020
luspatercept	Acceleron	Myelodysplastic syndrome; Thalassemia	SC	Submitted - BLA; Fast Track; Orphan Drug	04/03/2020
remimazolam	Cosmo	Anesthesia	IV	Submitted - NDA	04/03/2020
filgrastim (biosimilar to Amgen's Neupogen)	Adello	Neutropenia/leukopenia	IV, SC	Submitted - BLA	Pending
filgrastim (biosimilar to Amgen's Neupogen)	Apotex	Neutropenia/leukopenia	SC	Submitted - BLA	Pending
pegfilgrastim (biosimilar to Amgen's Neulasta)	Apotex	Neutropenia/leukopenia	SC	Submitted - BLA	Pending
2-hydroxypropyl-ß-cyclodextrin	Mallinckrodt	Niemann-Pick disease	Intrathecal	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
abametapir	Dr. Reddy's	Head lice (aged ≥ 6 months)	Topical	Phase 3 - NDA	TBD
abicipar pegol	Allergan	Wet AMD	Intraocular	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Coherus	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Fresenius	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Kyowa Hakko Kirin	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Momenta	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Mylan	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
albuterol (ProAir RespiClick®)	Teva	COPD	Inhaled	Phase 3 - sNDA	TBD
alferminogene tadenovec	Gene Biotherapeutics	Angina	Intracoronary	Phase 3 - BLA; Fast Track	TBD
alicaforsen	Atlantic	UC	Rectal	Phase 3 - NDA; Fast Track; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
allopregnanolone	Sage	MDD	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track	TBD
alpelisib	Novartis	Breast cancer	Oral	Phase 3 - NDA	TBD
alpha-glucosidase (human, recombinant)	Sanofi	Pompe disease	IV	Phase 3 - BLA	TBD
amikacin (Arikayce®)	Insmed	CF	Inhaled	Phase 3 - sNDA; Orphan Drug	TBD
andolast	Mylan	Asthma	Inhaled	Phase 3 - NDA	TBD
anifrolumab	AstraZeneca	SLE	IV	Phase 3 - BLA; Fast Track	TBD
anti-digoxin antibody	AMAG	Eclampsia/pre-eclampsia	IV	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
apalutamide (Erleada)	Janssen	Prostate cancer (metastatic, castration-resistant)	IV	Phase 3 - sNDA	TBD
astodrimer	Starpharma	Bacterial vaginosis (treatment & prevention)	Intravaginal	Phase 3 - Fast Track; QIDP	TBD
atezolizumab (Tecentriq)	Genentech	RCC; Breast cancer (triple-negative, in combination with nab-paclitaxel)	IV	Phase 3 - sBLA	TBD
avacopan	Chemocentryx	Antineutrophil cytoplasmic antibodies (ANCA) associated vasculitis	Oral	Phase 3 - NDA; Orphan Drug	TBD
avapritinib	Blueprint	GI stromal tumor	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
baclofen/naltrexone/sorbitol	Pharnext	Charcot-Marie-Tooth disease	Oral	Phase 3 - NDA; Orphan Drug	TBD
baricitinib (Olumiant®)	Eli Lilly	Atopic dermatitis	Oral	Phase 3 - sNDA	TBD
benralizumab (Fasenra®)	AstraZeneca	Nasal polyposis	SC	Phase 3 - sBLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Boehringer Ingelheim	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Kyowa Hakko Kirin	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bimatoprost SR	Allergan	Glaucoma/ocular hypertension	Ophthalmic	Phase 3 - NDA	TBD
bimekizumab	UCB	PSO	IV	Phase 3 - BLA	TBD
biotin (high-dose)	Medday	MS	Oral	Phase 3 - NDA	TBD
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease	Oral	Phase 3 - sNDA; Fast Track	TBD
brolucizumab	Novartis	Diabetic macular edema; Macular edema (due to retinal vein occlusion)	Intraocular	Phase 3 - BLA	TBD
budesonide HFA-MDI	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
budesonide/formoterol fumarate/glycopyrronium	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
budesonide/formoterol MDI	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
C1-esterase inhibitor (Cinryze®)	Shire	Hereditary angioedema	SC	Phase 3 - NDA	TBD
cabotegravir	GlaxoSmithKline	HIV-1 prevention (women)	IM	Phase 3 - NDA	TBD
calcipotriene/ betamethasone dipropionate	MC2	PSO	Topical	Phase 3 - 505(b)(2) NDA	TBD
cannabidiol (Epidiolex®)	GW	Tuberous sclerosis complex	Oral	Phase 3 - sNDA; Orphan Drug	TBD
cantharidin	Verrica	Molluscum contagiosum	Topical	Phase 3 - NDA	TBD
capmatinib	Novartis	NSCLC	Oral	Phase 3 - NDA	TBD
capsaicin	Centrexion	Osteoarthritis	Intra-articular	Phase 3 - NDA; Fast Track	TBD
casimersen	Sarepta	Duchenne muscular dystrophy	IV	Phase 3 - NDA	TBD
cefiderocol	Shionogi	HAP	IV	Phase 3 - NDA	TBD
cenicriviroc mesylate	Allergan	NASH	Oral	Phase 3 - NDA; Fast Track	TBD
cetuximab (Erbitux®)	Eli Lilly	Colorectal cancer (metastatic, BRAFV600E+, with binimetinib & cetuximab)	IV	Phase 3 - sBLA; Breakthrough Therapy; Fast Track	TBD
citrulline	Asklepion	Acute lung injury	IV	Phase 3 - NDA; Orphan Drug	TBD
clascoterone	Cassiopea	Acne	Topical	Phase 3 - NDA	TBD
CM-AT	Curemark	Autism spectrum disorders	Oral	Phase 3 - BLA; Fast Track	TBD
coversin	Akari	Paroxysmal nocturnal hemoglobinuria	SC	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
crizanlizumab	Novartis	Sickle cell anemia	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
CTP-modified human growth hormone	Opko	Growth hormone deficiency	SC	Phase 3 - BLA; Orphan Drug	TBD
cyclobenzaprine	Tonix	Post-traumatic stress disorder	SL	Phase 3 - 505(b)(2) NDA	TBD
dalcetrapib	Dalcor	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
daprodustat	GlaxoSmithKline	Anemia due to CKD (dialysis-independent & dialysis-dependent)	Oral, Topical	Phase 3 - NDA	TBD
darvadstrocel	Tigenix	CD	IV	Phase 3 - BLA; Orphan Drug	TBD
dasiglucagon	Zealand	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 - NDA; Orphan Drug	TBD
dehydrated human amnion chorion membrane	Mimedx	Achilles tendonitis; Plantar fasciitis	IV	Phase 3 - BLA	TBD
delafloxacin (Baxdela®)	Melinta	CAP	IV, Oral	Phase 3 - sNDA; QIDP	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
depatuxizumab mafodotin	Abbvie	Brain cancer	IV	Phase 3 - BLA; Orphan Drug	TBD
deramanido	Otsuka	Tuberculosis	Oral	Phase 3 - NDA	TBD
diacerein	Castle Creek	Epidermolysis bullosa	Topical	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
dianhydrogalactitol	Delmar	Brain cancer	IV	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
difelikefalin	Cara	Pruritus	IV	Phase 3 - NDA; Breakthrough Therapy	TBD
dinutuximab beta	EUSA	Neuroendocrine tumors	SC	Phase 3 - BLA; Orphan Drug	TBD
donaperminogene seltoplasmid	Viromed	Diabetic foot ulcers; Diabetic peripheral neuropathy	IM	Phase 3 - BLA; Regenerative Medicine Advanced Therapy	TBD
durvalumab (Imfinzi®)	AstraZeneca	SCLC	IV	Phase 3 - sBLA	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 - NDA; Fast Track	TBD
dust mite immunotherapy	Stallergenes	Allergic rhinitis	SL	Phase 3 - BLA	TBD
ebola vaccine	Merck	Ebola infection prevention	IM	Phase 3 - BLA; Breakthrough Therapy	TBD
efgartigimod	Argenx	Myasthenia gravis	IV	Phase 3 - BLA; Orphan Drug	TBD
eflapegrastim	Spectrum	Neutropenia/leukopenia	SC	Phase 3 - BLA	TBD
eflornithine/sulindac	Cancer Prevention	Familial adenomatous polyposis	Oral	Phase 3 - 505(b)(2) NDA; Fast Track; Orphan Drug	TBD
efpeglenatide	Hanmi	T2DM	SC	Phase 3 - NDA	TBD
elafibranor	Genfit	NASH	Oral	Phase 3 - NDA; Fast Track	TBD
elagolix (Orilissa®)	Abbvie	Uterine fibroids	Oral	Phase 3 - sNDA	TBD
empagliflozin (Jardiance®)	Boehringer Ingelheim	Diabetic nephropathy	Oral	Phase 3 - sNDA	TBD
enfortumab vedotin	Astellas	Bladder cancer	IV	Phase 3 - BLA; Breakthrough Therapy	TBD
EP-2101 vaccine	OSE Immunotherapeutics	NSCLC	SC	Phase 3 - BLA; Orphan Drug	TBD
epoetin alfa (biosimilar to Janssen's Procrit)	Novartis	Anemia due to CKD (dialysis-dependent)	IV, SC	Phase 3 - BLA	TBD
eptacog alfa (NovoSeven®)	Novo Nordisk	Factor VIII intolerance	IV	Phase 3 - sBLA	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 - NDA	TBD
estetrol/drospirenone	Mithra	Contraception	Oral	Phase 3 - NDA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Coherus	RA; PSO	SC	Phase 3 - BLA	TBD
fenfluramine (low-dose)	Zogenix	Dravet syndrome	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ferric maltol	Shield	Anemia due to CKD (dialysis-independent)	Oral	Phase 3 - NDA	TBD
ferric pyrophosphate (Triferic®)	Rockwell Medical	Anemia due to CKD (dialysis-dependent)	IV	Phase 3 - sNDA	TBD
fevipiprant	Novartis	Asthma	Oral	Phase 3 - NDA	TBD
fexapotide triflutate	Nymox	Benign prostatic hyperplasia	Intratumoral	Phase 3 - NDA	TBD
filgotinib	Gilead	RA; CD; UC	Oral	Phase 3 - NDA	TBD
fitusiran	Sanofi	Hemophilia A and B (with and without inhibitors)	SC	Phase 3 - NDA; Orphan Drug	TBD
fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®)	GlaxoSmithKline	Asthma	Inhaled	Phase 3 - sNDA	TBD
follitropin alfa (follow-on to EMD Serono's Gonal-F)	Finox	Reproductive disorder	SC	Phase 3 - 505(b)(2) NDA	TBD
follitropin alfa (follow-on to EMD Serono's Gonal-F®)	Allergan	Reproductive disorder	SC	Phase 3 - 505(b)(2) NDA	TBD
formoterol fumarate MDI	AstraZeneca	COPD	Inhaled	Phase 3 - 505(b)(2) NDA	TBD
fosmetpantotenate	Retrophin	Pantothenate kinase-associated neurodegeneration	IV	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
fostemsavir	GlaxoSmithKline	HIV-1 infection	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track	TBD
fusidic acid	Melinta	ABSSSI	Oral	Phase 3 - NDA; QIDP	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 - NDA	TBD
givosiran	Alnylam	Porphyria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
GLPG1690	Galapagos	Idiopathic pulmonary fibrosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
glucagon pump	Xeris	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 - NDA; Orphan Drug	TBD
glycopyrrolate HFA MDI	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
glycopyrrolate hydrofluoroalkane MDI	AstraZeneca	Asthma	Inhaled	Phase 3 - NDA	TBD
glycopyrronium bromide (Seebri® Neohaler®)	Sumitomo Dainippon	Asthma	Inhaled	Phase 3 - sNDA	TBD
grazoprevir/elbasvir (Zepatier®)	Merck	Hepatitis C infection (with CKD)	Oral	Phase 3 - sNDA; Breakthrough Therapy	TBD
GS010	Gensight	Leber's hereditary optic neuropathy	Intraocular	Phase 3 - BLA; Orphan Drug	TBD
guadecitabine	Otsuka	AML	SC	Phase 3 - NDA; Orphan Drug	TBD
guselkumab (Tremfya®)	Janssen	PsA	SC	Phase 3 - sBLA	TBD
HTT-ASO	Roche	Huntington's disease	Intrathecal	Phase 3 - NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
ibrexafungerp	Scynexis	Fungal infections (systemic and non-systemic)	IV, Oral	Phase 3 - NDA; Fast Track; Orphan Drug; QIDP	TBD
idasanutlin	Roche	AML	Oral	Phase 3 - NDA	TBD
idebenone	Santhera	Duchenne muscular dystrophy	Oral	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
immunoglobulin IV 10%	Octapharma	Thrombocytopenia	IV	Phase 3 - BLA	TBD
immunoglobulin IV 10%	Prometic Life	Primary immunodeficiencies	IV	Phase 3 - BLA	TBD
inclisiran	The Medicines Company	Dyslipidemia/ hypercholesterolemia	SC	Phase 3 - NDA; Orphan Drug	TBD
indacaterol maleate/ mometasone furoate	Novartis	Asthma	Inhaled	Phase 3 - NDA	TBD
indacaterol/ glycopyrronium bromide/ mometasone furoate	Novartis	Asthma	Inhaled	Phase 3 - NDA	TBD
inebilizumab	Viela	Neuromyelitis optica (Devic's syndrome)	IV	Phase 3 - BLA; Orphan Drug	TBD
infliximab (biosimilar to Janssen's Remicade)	Nichi-Iko	RA	IV	Phase 3 - BLA	TBD
insulin glargine (follow-on to Sanofi's Lantus)	Gan & Lee	T2DM	SC	Phase 3 - 505(b)(2) NDA	TBD
interferon-beta 1a	Faron	Acute respiratory distress syndrome (ARDS)	IV	Phase 3 - BLA; Fast Track	TBD
isatuximab	Sanofi	Multiple myeloma	IV	Phase 3 - BLA; Orphan Drug	TBD
KX01	Almirall Prodesfarma	Actinic keratoses	Oral	Phase 3 - NDA	TBD
lentiviral beta-globin gene transfer	Bluebird bio	Beta-thalassemia (transfusion-dependent)	IV	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
lentiviral vector hematopoietic stem cell gene therapy	Bluebird bio	Cerebral adrenoleukodystrophy	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
leronlimab	CytoDyn	HIV-1 infection	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
lisocabtagene maraleucel	Celgene	DLBCL	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug; Regenerative Medicine Advanced Therapy	TBD
L-lactic acid/citric acid/ potassium bitartrate	Evofem	Contraception; UTI	Intravaginal	Phase 3 - NDA; Fast Track; QIDP	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
lumasiran	Alnylam	Hyperoxaluria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
lumateperone	Intra-cellular Therapies	Bipolar disorder	Oral	Phase 3 - NDA	TBD
maribavir	Shire	Cytomegalovirus infection treatment	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
masitinib mesylate	AB Science	Alzheimer's disease; Asthma	Oral	Phase 3 - NDA	TBD
mavacamten	Myokardia	Obstructive hypertrophic cardiomyopathy	Oral	Phase 3 - NDA; Orphan Drug	TBD
mepolizumab (Nucala®)	GlaxoSmithKline	Nasal polyposis	SC	Phase 3 - sBLA	TBD
meropenem/vaborbactam (Vabomere®)	Melinta	HAP; Septicemia	IV	Phase 3 - sNDA; QIDP	TBD
metachromatic leukodystrophy gene therapy	Orchard	Metachromatic leukodystrophy	IV	Phase 3 - BLA; Orphan Drug	TBD
microbiota suspension	Rebiotix	Recurrent *Clostridium difficile* infection	Rectal	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
minocycline	Foamix	Rosacea	Topical	Phase 3 - 505(b)(2) NDA	TBD
mirikizumab	Eli Lilly	UC	SC	Phase 3 - BLA	TBD
mirvetuximab soravtansine	Immunogen	Ovarian cancer	IV	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
mitomycin	Urogen	Bladder cancer	Intravesical	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
molgramostim	Savara	Pulmonary alveolar proteinosis	Inhaled	Phase 3 - BLA; Orphan Drug	TBD
multipotent adult progenitor cells	Athersys	Ischemic stroke	IV	Phase 3 - BLA; Fast Track; Regenerative Medicine Advanced Therapy	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 - NDA	TBD
nitric oxide	AIT	Bronchiectasis	Inhaled	Phase 3 - NDA	TBD
nolasiban	Obseva	Female infertility	Oral	Phase 3 - NDA	TBD
obeticholic acid (Ocaliva®)	Intercept	NASH	Oral	Phase 3 -sNDA; Breakthrough Therapy	TBD
olaparib (Lynparza®)	AstraZeneca	Breast cancer (adjuvant treatment)	Oral	Phase 3 - sNDA; Breakthrough Therapy	TBD
oliceridine	Trevena	Acute pain	IV	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
olipudase alfa	Sanofi	Niemann-Pick disease	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
omalizumab (Xolair®)	Genentech	Nasal polyposis	SC	Phase 3 - sBLA	TBD
ondansetron ER (once daily)	Redhill	Gastroenteritis	Oral	Phase 3 - 505(b)(2) NDA	TBD
opicapone	Neurocrine Biosciences	Parkinson's disease	Oral	Phase 3 - NDA	TBD
oportuzumab monatox	Sesen Bio	Bladder cancer	Intravesical	Phase 3 - BLA; Fast Track	TBD
ozanimod	Celgene	CD; UC	Oral	Phase 3 - NDA	TBD
paclitaxel (micellar)	Oasmia	Ovarian cancer	IV	Phase 3 - NDA; Orphan Drug	TBD
palovarotene	Clementia	Fibrodysplasia ossificans progressiva	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
pegunigalsidase alfa	Chiesi	Fabry disease	IV	Phase 3 - BLA; Fast Track	TBD
PF-06651600	Pfizer	Alopecia areata	Oral	Phase 3 - NDA; Breakthrough Therapy	TBD
pimodivir	Janssen	Influenza treatment	Oral	Phase 3 - NDA; Fast Track	TBD
pineapple proteolytic enzymes extract	Mediwound	Burn Injury	Topical	Phase 3 - BLA; Orphan Drug	TBD
plasminogen (human)	Kedrion	Ligneous conjunctivitis	Ophthalmic	Phase 3 - BLA; Orphan Drug	TBD
plinabulin	Beyondspring	NSCLC; Neutropenia/ leukopenia	IV	Phase 3 - NDA	TBD
pollinex quattro grass	Allergy Therapeutics	Allergic rhinitis	SC	Phase 3 - BLA	TBD
QPI-1002	Quark	Delayed graft function; Kidney injury prevention following cardiac surgery	IV	Phase 3 - NDA	TBD
ranibizumab (biosimilar to Genentech's Lucentis®)	Santo	Wet AMD	Intraocular	Phase 3 - BLA	TBD
ravulizumab (Ultomiris®)	Alexion	Hemolytic uremic syndrome	IV, SC	Phase 3 - sBLA; Orphan Drug	TBD
relugolix	Myovant	Endometriosis; Uterine fibroids	Oral	Phase 3 - NDA	TBD
remestemcel-L	Mesoblast	GVHD	IV	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
reproxalap	Aldeyra	Congenital ichthyosis	Topical	Phase 3 - NDA; Orphan Drug	TBD
resmetirom	Madrigal	NASH	Oral	Phase 3 - NDA	TBD
ridinilazole	Summit	*Clostridium difficile*-associated diarrhea/ infection	Oral	Phase 3 - NDA; Fast Track; QIDP	TBD
rifabutin/amoxicillin/ pantoprazole	Redhill	*H. pylori* Infection	Oral	Phase 3 - NDA; Fast Track; QIDP	TBD
rimegepant	Biohaven	Migraine treatment	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
risankizumab	Abbvie	CD	SC	Phase 3 - BLA; Orphan Drug	TBD
risperidone	Braeburn	Schizophrenia	SC Implant	Phase 3 - 505(b)(2) NDA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Amgen	RA; CLL/ SLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis	IV	Phase 3 - BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Archigen Biotech	RA; CLL; NHL (indolent); Antineutrophil cytoplasmic antibodies associated vasculitis	IV, SC	Phase 3 - BLA	TBD
ropeginterferon alfa-2b	Pharmaessentia	Polycythemia vera	SC	Phase 3 - BLA; Orphan Drug	TBD
rovalpituzumab tesirine	Abbvie	SCLC	IV	Phase 3 - BLA; Orphan Drug	TBD
roxadustat	AstraZeneca	Anemia due to CKD (dialysis-independent & dialysis-dependent); Anemia due to oncology treatment	Oral	Phase 3 - NDA	TBD
RSV F nanoparticle vaccine	Novavax	Respiratory syncytial virus prevention	IM	Phase 3 - BLA; Fast Track	TBD
RVT-802 (postnatal thymus tissue transplant)	Enzyvant	Congenital athymia	TBD	Phase 3 - Breakthrough Therapy; Orphan Drug; Regenerative Medicine Advanced Therapy	TBD
sacubitril/valsartan (Entresto®)	Novartis	Heart failure (preserved ejection fraction)	Oral	Phase 3 - sNDA; Fast Track	TBD
samidorphan/olanzapine	Alkermes	Schizophrenia	Oral	Phase 3 - NDA	TBD
satralizumab	Roche	Neuromyelitis optica (Devic's syndrome)	SC	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
savolitinib	AstraZeneca	RCC	Oral	Phase 3 - NDA	TBD
sci-B-vac	VBI Vaccines	Hepatitis B virus prevention	IM	Phase 3 - BLA	TBD
seladelpar	Cymabay	Primary biliary cholangitis/hepatic fibrosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
selinexor	Karyopharm	Sarcoma	Oral	Phase 3 - NDA; Orphan Drug	TBD
selonsertib	Gilead	NASH	Oral	Phase 3 - NDA	TBD
semaglutide (Ozempic)	Novo Nordisk	Obesity	SC	Phase 3 - sNDA	TBD
serlopitant	Menlo	Pruritus associated with prurigo nodularis (PN)	Oral	Phase 3 - NDA; Breakthrough Therapy	TBD
setmelanotide	Rhythm	Obesity	SC	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
sodium oxybate (low-dose)	Jazz	Narcolepsy	Oral	Phase 3 - NDA	TBD
sodium oxybate (once-nightly dosing)	Avadel	Narcolepsy	Oral	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
sodium thiosulfate	Fennec	Chemotherapy-induced hearing loss	IV	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
sotagliflozin	Sanofi	T2DM	Oral	Phase 3 - NDA	TBD
sparsentan	Retrophin	Focal segmental glomerulosclerosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
spartalizumab	Novartis	Melanoma	IV	Phase 3 - BLA	TBD
sulopenem etzadroxil	Iterum	Uncomplicated UTI	IV, Oral	Phase 3 - NDA; QIDP	TBD
tanezumab	Pfizer	Osteoarthritis; Chronic low back pain, Cancer pain	IV	Phase 3 - BLA; Fast Track	TBD
tasimelteon (Hetlioz)	Vanda	Smith-Magenis syndrome	Oral	Phase 3 - sNDA	TBD
tecarfarin	Espero	Anticoagulation	Oral	Phase 3 - NDA	TBD
tenapanor	Ardelyx	Hyperphosphatemia	Oral	Phase 3 - NDA	TBD
teprasiran	Quark	Delayed graft function	IV	Phase 3 - NDA; Orphan Drug	TBD
teprotumumab	Horizon	Graves' ophthalmopathy/ orbitopathy	IV	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
terlipressin	Mallinckrodt	Hepatorenal syndrome	IV	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
tezepelumab	AstraZeneca	Asthma	IV, SC	Phase 3 - BLA	TBD
timapiprant	Chiesi	Asthma	Oral	Phase 3 - NDA	TBD
timbetasin	Regenerx	Ophthalmic wound healing; Dry eye syndrome	Topical	Phase 3 - NDA; Orphan Drug	TBD
tisagenlecleucel-t (Kymriah®)	Novartis	CLL/SLL	IV	Phase 3 - sBLA; Orphan Drug	TBD
tivanisiran	Sylentis	Dry eye	Topical	Phase 3 - NDA	TBD
tivozanib	Aveo	RCC	Oral	Phase 3 - NDA	TBD
tocilizumab (Actemra®)	Roche	Scleroderma	SC	Phase 3 - sBLA; Breakthrough Therapy	TBD
tonogenchoncel-L	Kolon Tissuegene	Osteoarthritis	Intra-articular	Phase 3 - BLA	TBD
tralokinumab	AstraZeneca	Atopic dermatitis	SC	Phase 3 - BLA	TBD
transcon PEG growth hormone	Ascendis	Growth hormone deficiency	SC	Phase 3 - BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Tanvex	Breast cancer; Gastric/ gastroesophageal cancer	IV	Phase 3 - BLA	TBD
trastuzumab deruxtecan	Daiichi Sankyo	Breast cancer	IV	Phase 3 - BLA; Breakthrough Therapy; Fast Track	TBD
TRC101	Tricida	Renal disease/Renal failure	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
treprostinil (patch pump)	Steadymed	PAH	SC	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
triamcinolone acetonide	Clearside	Uveitis	Intraocular	Phase 3 - 505(b)(2) NDA	TBD
ublituximab	TG	CLL/SLL	IV	Phase 3 - BLA; Orphan Drug	TBD
udenafil	Allergan	Erectile dysfunction	Oral	Phase 3 - NDA	TBD
upadacitinib	Abbvie	Atopic dermatitis; Axial spondyloarthritis; PsA; CD; UC	Oral	Phase 3 - NDA; Breakthrough Therapy	TBD
ursodeoxycholic acid	Retrophin	Primary biliary cholangitis	Oral	Phase 3 - NDA	TBD
ustekinumab (Stelara)	Janssen	SLE	IV, SC	Phase 3 - sBLA	TBD
vadadustat	Akebia	Anemia due to CKD (dialysis-independent & dialysis-dependent)	Oral	Phase 3 - NDA	TBD
valoctocogene roxaparvovec	Biomarin	Hemophilia A	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
vernakalant	Correvio	Atrial fibrillation	IV	Phase 3 - NDA	TBD
viaskin peanut	DBV	Peanut allergy (aged 4 to 11 years)	Transdermal	Phase 3 - BLA; Breakthrough Therapy; Fast Track	TBD
vibegron	Roivant	Overactive bladder	Oral	Phase 3 - NDA	TBD
vilanterol trifenatate	GlaxoSmithKline	Asthma; COPD	Inhaled	Phase 3 - NDA	TBD
vilaprisan	Bayer	Uterine fibroids	Oral	Phase 3 - NDA	TBD
viloxazine	Supernus	ADHD	Oral	Phase 3 - NDA	TBD
visomitin	Mitotech	Dry eye	Topical	Phase 3 - NDA	TBD
vocimagene amiretrorepvec	Tocagen	Brain cancer (malignant glioma; glioblastoma)	Intratumoral	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
volanesorsen	Akebia	Lipodystrophy	SC	Phase 3 - NDA; Orphan Drug	TBD
vonapanitase	Proteon	End-stage renal disease	Topical	Phase 3 - BLA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
vosoritide	Biomarin	Achondroplasia	SC	Phase 3 - NDA; Orphan Drug	TBD
voxelotor	Global Blood Therapeutics	Sickle cell anemia	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
vutrisiran	Alnylam	Transthyretin amyloid cardiomyopathy (ATTR-CM, wild type or hereditary); Transthyretin amyloid polyneuropathy	SC	Phase 3 - NDA; Orphan Drug	TBD
VX-445/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA; Fast Track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
VX-659/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA	TBD
zanubrutinib	Beigene	Waldenstrom macroglobulinemia	Oral	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
zolmitriptan (microneedle patch)	Zosano	Migraine treatment	Transdermal	Phase 3 - 505(b)(2) NDA	TBD

Complete Response Letter (CRL)/Withdrawn Drugs

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
apomorphine film	Sumitomo Dainippon	Parkinson's disease	SL	CRL	TBD
iclaprim	Motif Bio	ABSSSI	IV	CRL	TBD
meloxicam	Recro	Postsurgical pain	IV	CRL	TBD
metoclopramide	Evoke	Diabetic gastroparesis	Intranasal	CRL	TBD
midazolam	UCB	Seizure disorders (acute treatment)	Intranasal	CRL	TBD
rizatriptan film	Intelgenx	Migraine treatment	Oral transmucosal	CRL	TBD
samidorphan/buprenorphine	Alkermes	MDD	SL	CRL	TBD
sotagliflozin	Sanofi	T1DM	Oral	CRL	TBD

GLOSSARY

ABSSSI Acute Bacterial Skin and Skin Structure Infection

ADHD Attention Deficit Hyperactivity Disorder

ALK Anaplastic Lymphoma Kinase

ALL Acute Lymphoblastic Leukemia

ALT Alanine Transaminase

AMD Age-Related Macular Degeneration

AML Acute Myeloid Leukemia

ANDA Abbreviated New Drug Application

ARC20 American College of Rheumatology 20% Improvement

ARC50 American College of Rheumatology 50% Improvement

ARC70 American College of Rheumatology 70% Improvement

AS Ankylosing Spondylitis

AST Aspartate Aminotransferase

BED Binge Eating Disorder

BLA Biologics License Application

BsUFA Biosimilar User Fee Act

CAP Community Acquired Pneumonia

CD Crohn's Disease

CDC Centers for Disease Control and Prevention

CF Cystic Fibrosis

CHF Congestive Heart Failure

CI Confidence Interval

CKD Chronic Kidney Disease

CLL Chronic Lymphocytic Leukemia

CNS Central Nervous System

COPD Chronic Obstructive Pulmonary Disease

CRC Colorectal Cancer

CRL Complete Response Letter

CV Cardiovascular

CVD Cardiovascular Disease

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

DEA Drug Enforcement Administration

DLBCL Diffuse Large B Cell Lymphoma

DMARD Disease Modifying Antirheumatic Drug

DPP-4 Dipeptidyl Peptidase 4

DR Delayed-Release

EDSS Expanded Disability Status Scale

EGFR Epidermal Growth Factor Receptor

ER Extended-Release

FDA Food and Drug Administration

FLT3 FMS-Like Tyrosine Kinase-3

GI Gastrointestinal

GLP-1 Glucagon-Like peptide-1

GVHD Graft versus Host Disease

H Half

HAP Healthcare-Associated Pneumonia

HAM-D Hamilton Depression Rating Scale

HbA1c Hemoglobin A1c

HCC Hepatocellular Carcinoma

HCP Healthcare Professional

HCV Hepatitis C Virus

HER Human Epidermal Growth Factor Receptor

HER2 Human Epidermal Growth Factor Receptor 2

HFA Hydrofluoroalkane

HIT Heparin Induced Thrombocytopenia

HIV-1 Human Immunodeficiency-1 Virus

HTN Hypertension

HR Hazard Ratio

IBS Irritable Bowel Syndrome

IM Intramuscular

IV Intravenous

JIA Juvenile Idiopathic Arthritis

LDL-C Low-Density Lipoprotein Cholesterol

MADRS Montgomery–Åsberg Depression Rating Scale

MDD Major Depressive Disorder

MDI Metered Dose Inhaler

MRSA Methicillin-Resistant Staphylococcus Aureus

MS Multiple Sclerosis

N/A Not Applicable

NASH Non-Alcoholic Steatohepatitis

NDA New Drug Application

NHL Non-Hodgkin Lymphoma

NSAID Non-Steroidal Anti-Inflammatory Drug

NSCLC Non-Small Cell Lung Cancer

ODT Orally Disintegrating Tablet

OS Overall Survival

PAH Pulmonary Arterial Hypertension

PARP poly(ADP-ribose) polymerase

PASI 50 Psoriasis Area and Severity Index ≥ 50%

PASI 70 Psoriasis Area and Severity Index ≥ 70%

PASI 90 Psoriasis Area and Severity Index ≥ 90%

PCI Percutaneous Coronary Intervention

PD-1 Programmed Death Protein 1

PD-L1 Programmed Death-Ligand 1

PDUFA Prescription Drug User Fee Application

PFS Progression-Free Survival

PGA Physicians Global Assessment

PsA Psoriatic Arthritis

PSO Plaque Psoriasis

PTCA Percutaneous Transluminal Coronary Angioplasty

Q Quarter

QIDP Qualified Infectious Diseases Product

QOL Quality of Life

RA Rheumatoid Arthritis

RCC Renal Cell Carcinoma

REMS Risk Evaluation and Mitigation Strategy

SL Sublingual

sBLA supplemental Biologics License Application

SC Subcutaneous

SCCHN Squamous Cell Cancer of the Head and Neck

SCLC Small Cell Lung Cancer

SCT Stem Cell Transplant

SGLT Sodium-Glucose coTransporter

SLE Systemic Lupus Erythematosus

SLL Small Lymphocytic Lymphoma

sNDA supplemental New Drug Application

SOC Standard of Care

sPGA Static Physicians Global Assessment

SR Sustained-Release

SSRI Selective Serotonin Reuptake Inhibitors

SNRI Serotonin and Norepinephrine Reuptake Inhibitors

SSSI Skin and Skin Structure Infection

T1DM Type 1 Diabetes Mellitus

T2DM Type 2 Diabetes Mellitus

TBD To Be Determined

TNFα Tumor Necrosis Factor-alpha

UA Unstable Angina

UC Ulcerative Colitis

US United States

UTI Urinary Tract Infection

WHO World Health Organization

XR Extended-Release

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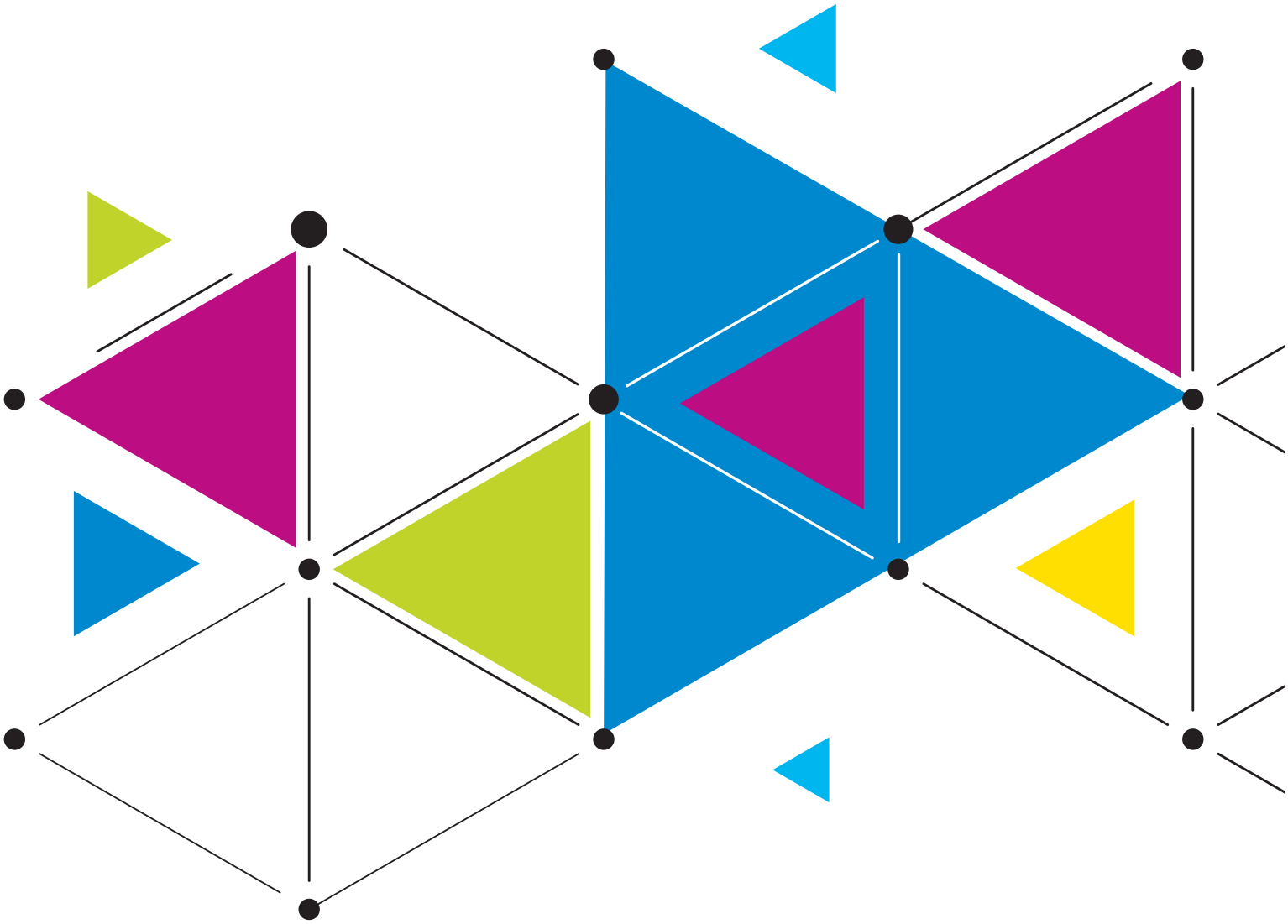

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