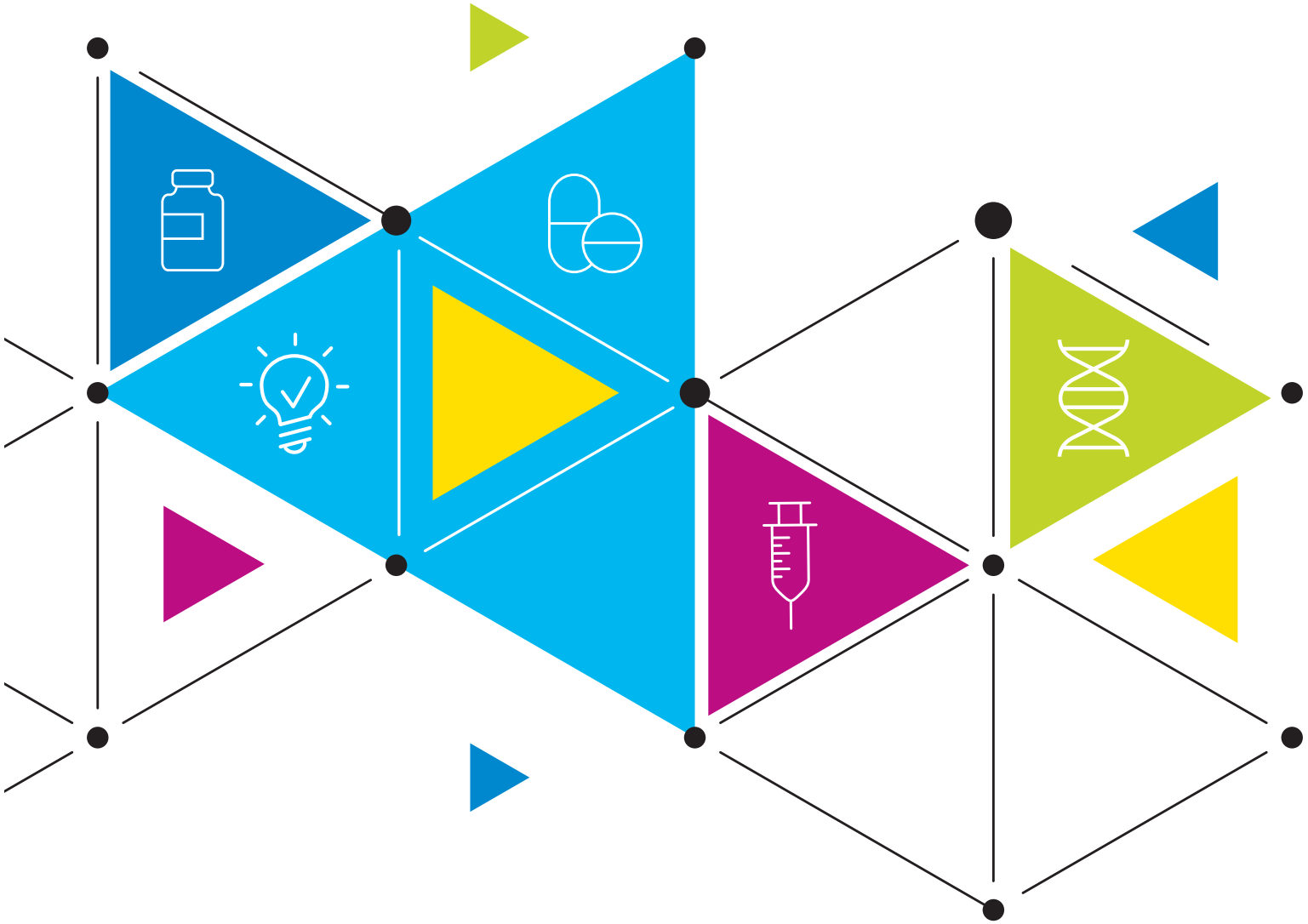
JANUARY 2019

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.

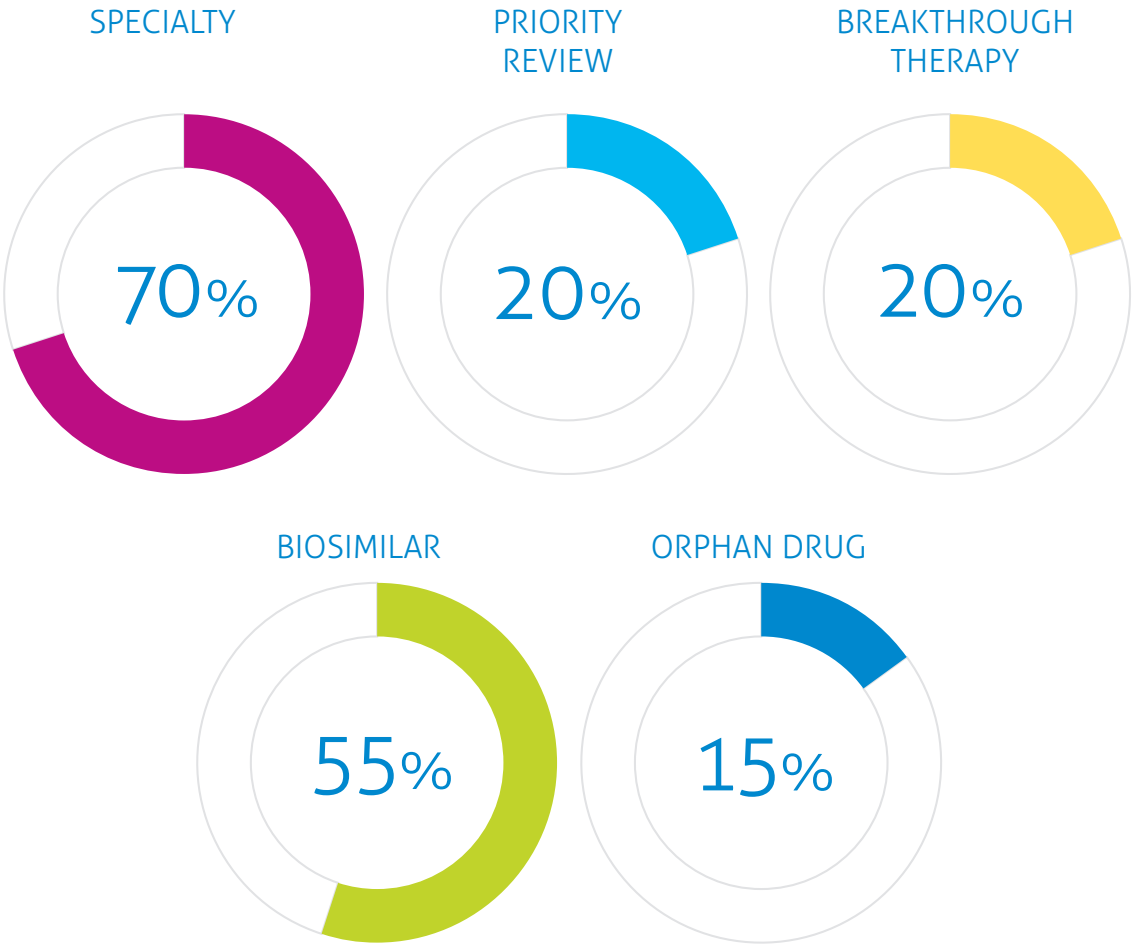
In order to assist payers with assessing the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Primarily complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected annual US sales through the year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

Last year, the US FDA set an all-time record of 59 novel drug approvals, surpassing the 46 novel drugs approved in 2017. The earlier part of this year has been off to a slow start due to the partial government shutdown. During the lapse period, the FDA was unable to accept any regulatory submissions that may have required a user fee payment. As a result, there could be delays to anticipated approvals in 2019. 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 women's health. Moreover, sprouting products for Alzheimer's disease, hemophilia, infectious diseases, hematology/oncology, and ophthalmology, 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.

Immunology

AR101 *oral*

Aimmune

PROPOSED INDICATIONS

Peanut allergy in children 4 to 11 years of age

CLINICAL OVERVIEW

AR101 is a biologic oral immunotherapy consisting of defatted roasted peanut flour with a characterized allergen profile.

The phase 3 PALISADE trial enrolled 551 peanut-allergic patients aged 4 to 55 years (n=496; four to 17 years). After 6 months of maintenance treatment, 67.2% of AR101-treated patients were able to consume ≥ 600 mg of peanut protein (equivalent to ≥ 2 peanuts) without reaction compared to 4% of patients treated with placebo. Among patients aged 4 to 17 years, 34.7% in the AR101 group experienced mild adverse events compared with 50% in the placebo group; 59.7% and 44.4% of the participants, respectively, experienced a moderate event; and 4.3% and 0.8%, respectively, experienced a severe event. One case of anaphylaxis was reported in the AR101 group during maintenance therapy. Efficacy was not shown in participants ≥ 18 years of age.

Contents of the AR101 capsule are mixed with pudding or applesauce and consumed orally. The dose is titrated under the supervision of an HCP over 2 weeks to a target maintenance dose of 300 mg daily. Dosing is intended to continue indefinitely; AR101 is not a curative therapy.

PLACE IN THERAPY

Peanut allergy is a public health concern with a significant burden for patients and caregivers. Approximately 2% of children in the US suffer from peanut allergy and this figure is rising. Reaction to peanut exposure varies from mild skin and/or GI symptoms to severe angioedema and anaphylaxis. When accidental peanut exposure occurs, antihistamines can manage mild to moderate reactions, but patients must carry an epinephrine auto-injector to treat severe reactions.

There is currently no FDA-approved treatment for peanut allergy. The main approach to managing food allergy is to avoid the offending allergen. Conversely, the American Academy of Pediatrics (AAP) and the National Institute of Allergy and Infectious Diseases (NIAID) support early introduction of peanut protein to prevent peanut allergy in infants who are at increased risk of developing peanut allergy (e.g., history of severe eczema and/or egg allergy).

If approved, AR101 will provide a consistent dose of peanut allergen to increase a patient's tolerance to accidental peanut exposure and reduce the risk of serious allergic reactions. Regeneron's monoclonal antibody dupilumab (Dupixent®) is being investigated in combination with AR101 to desensitize patients with peanut allergy. DBV's pipeline viaskin peanut transdermal patch formulation is in late-stage development for peanut allergy. Moreover, the potential approval of agents for peanut allergies sets the stage for the development of new drugs for other food allergies.

FDA APPROVAL TIMELINE

August 21, 2019

✓ Breakthrough Therapy ✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$7	$90	$280	$618	$953

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

Women's Health

bremelanotide *SC*

AMAG

PROPOSED INDICATIONS

Hypoactive sexual desire disorder (HSDD) in premenopausal women

CLINICAL OVERVIEW

Bremelanotide is a melanocortin 4 receptor agonist that acts on the MC1 and MC4 receptors, which regulate sexual behavior. Its proposed mechanism of action balances inhibitory and excitatory neural pathways in the brain to restore sexual desire. Bremelanotide is intended to be used in anticipation of a sexual encounter.

Two pivotal, randomized, double-blind, placebo-controlled RECONNECT trials enrolled a total of 1,202 premenopausal women (≥ 18 years old; mean age 39 years) with HSDD and no psychological, gynecological, or urological conditions suspected to contribute to the sexual dysfunction. In both trials, after 24 weeks of treatment, bremelanotide demonstrated statistically significant improvement in desire, as measured by the Female Sexual Function Index – Desire (FSFI-D) domain, and decrease in associated distress, as measured by the Female Sexual Distress Scale – Desire/Arousal/Orgasm (FSDS-DAO) Desire score. While many secondary endpoints were met and both studies reported improvement in the number of satisfying sexual events (SSEs), the difference between bremelanotide and placebo in number of SSEs was not statistically significant (64.4% versus 50.9% in Study 301 and 64.8% versus 47.3% in Study 302, respectively). The most common adverse effects were transient mild nausea, flushing, and headache.

Bremelanotide was studied at a dose of 1.75 mg taken as-desired prior to a sexual encounter; no more than 1 dose was taken every 24 hours. Bremelanotide was self-administered SC via an auto-injector.

PLACE IN THERAPY

HSDD is one facet of female sexual disorder. It occurs in approximately 5% to 13% of women, with a peak incidence at around 40 to 60 years of age. In younger women, HSDD is frequently associated with patient factors, such as dysfunctional relationships, chronic disease, depression, gynecologic disorders, and use of certain medications (e.g., SSRIs). Diagnosis is usually made when symptoms cause distress or negatively impact relationships.

Treatment of female sexual dysfunction should be individualized to address underlying physical, psychological, and relational factors. It can include psychotherapy and pharmacotherapy (estrogen, short-term testosterone, antidepressants [e.g., bupropion, SSRI]). However, currently available pharmacologic therapies for female sexual dysfunction have limited efficacy and are associated with side effects and potential risks. The only FDA-approved medication for HSDD in premenopausal women is the oral once-daily serotonin agonist/antagonist, flibanserin (Addyi®). Flibanserin is taken at bedtime due to its sedative affects and carries boxed warnings for alcohol consumption, hypotension, and syncope.

If approved, bremelanotide, a first-in-class melanocortin agonist, will provide a new mechanism and route of administration to treat HSDD in premenopausal women. Unlike flibanserin, it is administered on an as-desired basis and can be safely used concurrently with alcohol.

FDA APPROVAL TIMELINE

June 21, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$5	$34	$74	$115	$129

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

esketamine *intranasal*

Janssen

PROPOSED INDICATIONS

Treatment-resistant depression (TRD) in adults

CLINICAL OVERVIEW

The N-methyl-D-aspartate (NMDA) receptor may play a role in the delayed response of monoaminergic antidepressants. Esketamine blocks this receptor and may lead to a more rapid antidepressant effect.

The SUSTAIN-1 clinical trial enrolled adults (n=705) with TRD (<25% response to at least 1 antidepressant treatment) without psychotic features. In patients in stable remission, treatment beyond 16 weeks with flexible-dosed esketamine led to a 51% reduction in risk of relapse compared to placebo (26.7% versus 45.3%, respectively; p=0.003). The most common adverse effects (≥ 20%) were dizziness, dissociation, nausea, and headache. Although not powered to measure efficacy, the open-label, long-term SUSTAIN-2 study (n=802) suggested sustained response for up to 52 weeks. The TRANSFORM-2 study (n=223) reported statistically significant improvement in MADRS score with flexible-dosed esketamine at day 28; however, in TRANSFORM-3 (n=138), at day 28, it narrowly missed statistical significance in patients aged ≥ 65 years (initial dose 28 mg). Furthermore, in TRANSFORM-1 (n=346), the change in MADRS total score at day 28 was not statistically different with fixed-doses of 84 mg esketamine compared to placebo. The phase 2 SYNAPSE trial suggests onset of action begins within hours of starting esketamine treatment. Transient dissociative symptoms and elevations in blood pressure were reported shortly after administration and resolved within 2 hours.

In all studies, esketamine was given in combination with a newly initiated oral antidepressant (duloxetine, escitalopram, sertraline, or venlafaxine ER). Esketamine was administered intranasally twice weekly for 4 weeks. It was then administered once weekly or once every other week based on depressive symptoms. Doses of 56 mg or 84 mg were studied in phase 3 trials; a starting dose in patients ≥ 65 years of age was 28 mg. In SUSTAIN-1, esketamine was self-administered.

PLACE IN THERAPY

In 2016, an estimated 10.3 million adults in the US experienced severe impairment from depression. Approximately 30% of patients with MDD are resistant to currently available medications. While there is no standardized definition for TRD, it is usually identified when symptoms do not resolve after at least 2 adequate courses of treatment.

Typically, SSRIs, SNRIs, mirtazapine, bupropion, or cognitive behavioral therapy (CBT) are initial treatments for depression. In patients who do not respond, addition of or switching to another medication (antidepressant, antipsychotic) with a different mechanism of action and/or CBT may provide benefit.

Esketamine nasal spray plus an oral antidepressant is associated with a rapid reduction of depressive symptoms and delayed time to relapse. It is the S-enantiomer of ketamine, a drug linked to recreational use due to its dissociative effects. Dissociative effects of esketamine may decrease with continued use.

Esketamine's rapid onset of action and infrequent dosing schedule may make it an attractive option for the maintenance treatment of TRD; however, if approved, its side effect profile and abuse potential may limit its use to the acute treatment setting and restrict its availability through a REMS program.

FDA APPROVAL TIMELINE

May 3, 2019

✓ Breakthrough Therapy ✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$48	$253	$433	$599	$752

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

iclaprim *IV*

Motif Bio

PROPOSED INDICATIONS

Acute bacterial skin and skin structure infections (ABSSSI)

CLINICAL OVERVIEW

Iclaprim is a bacterial dihydrofolate reductase (DHFR) inhibitor with broad spectrum of activity against Gram-positive bacteria.

Pooled data from the REVIVE-1 and REVIVE-2 trials (total n=1,190) demonstrated noninferiority (margin 10%) of iclaprim compared to vancomycin for the treatment of ABSSSI (lesion size ≥ 75 cm^2). This was demonstrated by an early clinical response (ECR) of at least a 20% reduction in lesion size 48 to 72 hours after initiating therapy. Across both studies, an ECR of 79.6% was reported with iclaprim and 78.8% with vancomycin, including methicillin-resistant *S. aureus* (MRSA) infections. The most common adverse effects with both drugs were headache and nausea. Elevated serum creatinine (SCr) was reported in 7 patients treated with vancomycin, including SCr ≥ 3 times upper limit of normal that indicates potential nephrotoxicity; no patients treated with iclaprim experienced elevated SCr. In pooled data from the ASSIST trials, similar ECRs were seen with iclaprim (73.6%) and linezolid (72.5%) in patients with complicated bacterial skin and skin structure infections (cBSSSI), including MRSA infections.

In the REVIVE trials, IV doses of iclaprim 80 mg every 12 hours and vancomycin 15 mg/kg every 12, 24, or 48 hours based on creatinine clearance were administered for 5 to 14 days. In the ASSIST trials, iclaprim 0.8 mg/kg and linezolid 600 mg were administered IV every 12 hours for 10 to 14 days.

PLACE IN THERAPY

ABSSSI is a common cause for hospitalization of adults in the US. ABSSSIs require prompt antibacterial treatment to prevent potentially life-threatening infections in bones, joints, surgical wounds, blood, heart valves, and lungs. Resistance to available antibiotics, increasing virulence, and presence of comorbidities (e.g., diabetes, obesity) may make selection of an appropriate antibiotic challenging.

Intravenous therapy should be considered based on severity of clinical presentation and patient comorbidities. IV therapy is warranted with suspected systemic infection, rapid progression of erythema, clinical progression after 48 hours of oral antibiotic therapy, patient inability to take oral therapy, and when the affected area is near an indwelling medical device. Vancomycin is the standard of care if MRSA is confirmed.

Iclaprim is a bactericidal agent that exhibits good tissue penetration. It was designed to overcome trimethoprim resistance with increased potency without the need for co-administration of a sulphonamide. Iclaprim is potent against a broad spectrum of Gram-positive bacteria, including MRSA and *S. aureus* that exhibit reduced susceptibility (VISA) or resistance (VRSA) to vancomycin. It is also active against several Gram-negative pathogens, such as *Haemophilus influenzae* and *Moraxella catarrhalis*. Iclaprim has demonstrated non-inferiority to vancomycin and linezolid. Unlike vancomycin, no cases of elevated SCr were reported with iclaprim. Iclaprim is also being studied in hospital-acquired and ventilator-associated bacterial pneumonias. Based on available information, it appears that no oral formulation of iclaprim is currently in development.

FDA APPROVAL TIMELINE

February 13, 2019

✓ Fast Track ✓ Priority Review ✓ Qualified Infectious Disease Product

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$11	$50	$114	$207	$317

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

Pain Management

NKTR-181 *oral*

Nektar Therapeutics

PROPOSED INDICATIONS

Moderate to severe chronic low back pain in opioid-naïve adults

CLINICAL OVERVIEW

NKTR-181 is a full mu-opioid analgesic that uses a proprietary polymer conjugate technology that allows the drug to enter the CNS at a considerably lower rate compared to existing opioid medications and results in little to no euphoria, respiratory depression, or sedation.

In the 12-week, phase 3, randomized, withdrawal design SUMMIT-07 trial, statistically significant analgesia was experienced in opioid-naïve patients (n=610) with moderate-to-severe chronic low back pain treated with NKTR-181 (titrated to 100 mg to 400 mg twice daily) compared to placebo. At 12 weeks, pain reductions from screening by ≥ 30% and ≥ 50% occurred in 71.2% and 51.1% of NKTR-181-treated patients, respectively, versus 57.1% and 37.9%, respectively, for those treated with placebo ($p \leq 0.001$). Abuse potential was similar between the groups, as demonstrated using the Misuse, Abuse, Diversion Drug Event Reporting System (MADDERS®). About 5% of patients in each group reported taking more medication than instructed (intentionally or unintentionally) for a therapeutic purpose. Withdrawal symptoms were reported in 2.9% of patients taking NKTR-181 and 2.1% taking placebo. Abuse potential of NKTR-181 was also evaluated in the phase 3, double-blind, crossover human abuse potential (HAP) study using a bi-polar Visual Analogue Scale (VAS) and Drug Effects Questionnaire (DEQ). Healthy nondependent recreational drug users (n=54) reported that the incidence of "drug-liking" and "feeling high" with single-dosed NKTR-181 (400 mg [therapeutic dose], 600 mg, 1,200 mg) was similar to placebo and was significantly lower than single-dosed oxycodone (40 mg and 60 mg) ($p<0.0001$, all comparisons).

PLACE IN THERAPY

Low back pain is a common cause of disability in adults. Opioid analgesics are often prescribed to manage pain. As the opioid crisis continues, an average 130 deaths occur in the US each day from opioid overdose. The FDA encourages the development of abuse-deterrent and tamper-resistant formulations. If approved, NKTR-181 will be the first full mu-opioid receptor agonist designed to provide potent pain relief without the inherent euphoria that contributes to abuse and addiction, and without the respiratory and CNS side effects that are seen with conventional opioids. This is achieved with a novel molecular structure of NKTR-181, which slows penetration across the blood-brain barrier in order to diminish the dopamine release that is responsible for euphoric effects. Historically, abuse-deterrent strategies relied on reformulation of current rapid-acting opioids, which can be altered to achieve euphoric effects. NKTR-181 is not a prodrug or a reformulation, and there is no known chemical or physical means to alter the low permeability across the blood-brain barrier; therefore, attempts to tamper with the formulation to increase rate of absorption will not lead to euphoria. In addition, its 12-hour elimination half-life allows for twice-daily dosing. The initial indication of NKTR-181 is expected to be for use in opioid-naïve individuals; however, NKTR-181 is in clinical trials in opioid-experienced patients, including in a long-term safety study (SUMMIT-LTS).

FDA APPROVAL TIMELINE

May 28, 2019

✓ Fast Track

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$97	$107	$118	$128	$138

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

onasemnogene abeparvovec *IV*

Novartis

PROPOSED INDICATIONS

Spinal muscular atrophy (SMA) type 1

CLINICAL OVERVIEW

Spinal muscular atrophy (SMA) is a rare, debilitating disease characterized by progressive motor function decline and muscular atrophy while sparing cognitive abilities. It is caused by a missing or defective survival motor neuron (SMN)-1 gene resulting in lack of SMN protein needed to maintain spinal cord and lower brain stem motor neurons. Patients with SMA type 1 typically do not achieve motor milestones and require nutritional ± respiratory support by age 12 months. Onasemnogene abeparvovec is a gene therapy that delivers a functioning copy of the SMN1 gene without modifying the existing DNA.

In the phase 1/2, open-label START trial 12 patients with SMA type 1 received a 1-time IV infusion of onasemnogene abeparvovec. At 24 months after the dose, all 12 patients were alive and did not need permanent ventilation; 7 infants did not require non-invasive ventilation (NIV). Eleven infants could be fed orally. Eleven infants could speak, had full head control, and could sit unassisted for ≥ 5 seconds, 9 infants could sit unassisted for ≥ 30 seconds. Two infants could walk independently. Mean number and duration of hospitalizations were lower with onasemnogene abeparvovec compared to the natural history of the disease (number, 2.1 versus 4.2, respectively; duration, 3.7 versus 13 days, respectively). Sustained and new motor milestones were observed with long-term follow-up, including 4 years post-dose. During long-term follow-up, an additional 2 patients achieved unassisted sitting for ≥ 30 seconds and 2 infants could stand with support. The most commonly reported adverse effect was transient asymptomatic elevated liver enzymes. A phase 3 study (STR1VE) is ongoing.

PLACE IN THERAPY

SMA is the leading genetic cause of infant mortality. Currently, 5 types of SMA have been identified and vary by age of onset, severity, and prognosis. SMA type 1 is the most common phenotype, reported in about 1 in 10,000 newborns. Onset is soon after birth to 6 months of age.

Onasemnogene abeparvovec is a gene replacement therapy. It crosses the blood-brain barrier to provide a rapid onset and lasting therapeutic effect for treating SMA type 1. If approved, it will be the first gene replacement therapy that requires a 1-time IV dose used as part of a multidisciplinary approach to treat SMA type 1. In December 2016, the SMN2-directed antisense oligonucleotide nusinersen (Spinraza®) became the first disease-modifying therapy to treat SMA (all types) in pediatrics and adults and is administered intrathecally as maintenance doses every 4 months. Another agent in late-stage development for SMA includes Novartis' branaplam, an SMN2 splicing modulator for SMA type 1.

Onasemnogene abeparvovec is also being studied (SPR1NT) in presymptomatic infants < 6 months old with SMA types 1-3, which has the potential to prevent SMA occurrence. Moreover, while timing of therapy initiation is critical in reaping the maximal effect, it is unknown if intervening at advanced disease stages can provide benefit. Intrathecal administration of onasemnogene abeparvovec to treat SMA type 1 in older patients is being studied as well. While use of nusinersen after administration of onasemnogene abeparvovec has been reported, clinical benefit and risk of using nusinersen following onasemnogene abeparvovec have not been established.

FDA APPROVAL TIMELINE

May 2019

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

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$185	$508	$759	$900	$962

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

Oncology

quizartinib *oral*

Daiichi Sankyo

PROPOSED INDICATIONS

Treatment of adults with relapsed/refractory (R/R) acute myeloid leukemia (AML) with FLT3-ITD mutation

CLINICAL OVERVIEW

The fms-like tyrosine kinase (FLT3) receptor plays a key role in hematopoiesis. FLT3 receptor mutations, including internal tandem duplication (ITD), promote AML cell survival. Quizartinib is an oral multi-tyrosine kinase inhibitor of the platelet-derived growth factor receptor, KIT/c-KIT, and FLT3.

The phase 3, open-label, QuANTUM-R study compared monotherapy with oral quizartinib to salvage chemotherapy in 367 adults with FLT3-ITD R/R AML following standard first-line AML therapy with or without hematopoietic SCT. After a median follow-up of 23.5 months, median overall survival (OS) with quizartinib was 6.2 months compared to 4.7 months with chemotherapy (HR, 0.76; 95% CI, 0.58-0.98; p=0.1071). Median event-free survival (EFS) time was 6 weeks with quizartinib and 3.7 weeks with chemotherapy (HR, 0.9; 95% CI, 0.7-1.16; p=0.1071). Composite complete remission (CRc) rate was 48% with quizartinib and 27% with chemotherapy (nominal p=0.0001) and the CRc duration was 12.1 versus 5 weeks, respectively. Rate of SCT was 32% versus 12%, respectively (nominal p=0.0001). The most commonly reported serious (grade ≥ 3) adverse drug reactions (> 20%) were infection and febrile neutropenia.

Quizartinib was studied at oral doses of 60 mg once daily with a lead-in dose of 30 mg.

PLACE IN THERAPY

It is estimated that 19,520 new cases of AML were diagnosed in the US in 2018. Intensive chemotherapy with or without allogeneic hematopoietic SCT results in cure in about 30% to 40% of AML cases. Poor prognosis is seen particularly in patients with FLT3 mutations. FLT3-ITD mutations are found in about 30% of patients with AML. Treatment for R/R AML includes cytotoxic regimens.

If approved, quizartinib could compete with Astellas' once-daily oral kinase inhibitor gilteritinib (Xospata™) that was approved in November 2018 and investigational crenolanib in the FLT3-ITD mutation-positive R/R AML arena. Furthermore, gilteritinib targets AXL and tyrosine kinase domain (TKD) and, therefore, may bypass resistance mechanisms to FLT3 inhibitors. Gilteritinib and crenolanib are also in phase 3 trials as maintenance therapy following induction/consolidation chemotherapy in newly-diagnosed patients with FLT3-ITD-positive AML. Gilteritinib use is also being evaluated after allogeneic SCT.

FDA APPROVAL TIMELINE

May 25, 2019

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

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$23	$74	$116	$148	$175

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

Oncology

selinexor *oral*

Karyopharm

PROPOSED INDICATIONS

Penta-refractory multiple myeloma (MM)

CLINICAL OVERVIEW

Cancer cells overexpress nuclear export protein exportin 1 (XPO1) causing transfer of tumor suppressor proteins (TSPs) from the cell nucleus. The decrease of TSPs allows proliferation of damaged DNA. Selinexor is a selective inhibitor of nuclear export (SINE) compound that blocks XPO1, ultimately leading to tumor cell death while largely sparing normal cells.

The phase 1/2, single-arm STORM study enrolled 122 patients with MM who were refractory after a median of 7 prior treatment regimens. Two patients had prior CAR-T therapy, and 2 patients had prior hematopoietic SCT. Treatment with selinexor lead to an overall response rate (ORR) of 26.2%, including 2 stringent complete responses (sCRs), which were negative for minimal residual disease, 6 very good partial responses (VGPRs), and 24 partial responses (PRs). Both patients who had relapsed after CAR-T therapy achieved PRs. Median OS was 8.6 months, with a median progression-free survival (PFS) of 3.7 months and a median duration of response (DOR) of 4.4 months. The most common treatment-related adverse effects were cytopenias and GI effects, which were managed primarily with dose adjustments and/or supportive care. No significant major organ toxicities were reported. One treatment-related death occurred.

Selinexor was administered orally as 80 mg twice weekly in combination with low-dose dexamethasone.

PLACE IN THERAPY

Over 32,000 new cases of MM are predicted in the US for 2019, and nearly 13,000 deaths could occur due to the condition. Age at diagnosis is usually around 70 years. Newly diagnosed MM responds to cytotoxic chemotherapy; however, eventual treatment-resistant relapse usually occurs. During the past decade, the introduction of more effective, less toxic treatments, including immunomodulatory drugs, proteasome inhibitors, monoclonal antibodies, and histone deacetylase (HDAC) inhibitors, has brought significant improvements in survivial. Nonetheless, unmet medical need still exists.

If approved, selinexor will be a first-in-class transport protein exportin-1 (XPO1) inhibitor for the treatment of MM. In general, patients with penta-refractory myeloma have failed the IV administered proteasome inhibitors (bortezomib [Velcade®] and carfilzomib [Kyprolis®]), the oral immunomodulatory drugs (lenalidomide [Revlimid®] and pomalidomide [Pomalyst®]), and the IV anti-CD38 monoclonal antibody, daratumumab (Darzalex®), as well as alkylating agents. This leaves remaining options as the oral agents, ixazomib (Ninlaro®), which belongs to a class of drug that the patient has previously failed, and panobinostat (Farydak®), a HDAC inhibitor that is associated with serious toxicities (diarrhea, cardiac). Selinexor's place in therapy for refractory MM may depend on its side effect profile relative to panobinostat. Selinexor is also being studied in combination with a proteasome inhibitor plus dexamethasone, which could reach a broader patient base with refractory MM, and for the treatment of several solid tumors.

FDA APPROVAL TIMELINE

April 5, 2019

✓ Fast Track ✓ Orphan Drug ✓ Priority Review

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$27	$113	$237	$365	$475

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

turoctocog alfa pegol *IV*

Novo Nordisk

PROPOSED INDICATIONS

Hemophilia A

CLINICAL OVERVIEW

Turoctocog alfa pegol is a long-acting recombinant factor VIII (FVIII) replacement product.

The PATHFINDER clinical program evaluated turoctocog alfa pegol in 252 adults and pediatrics with hemophilia A. In PATHFINDER-2, after treatment for up to 21 months, the median annualized bleeding rate (ABR) was 1.3 episodes in adults (n=175) treated prophylactically with turoctocog alfa pegol 50 IU/kg every 4 days versus 30.9 episodes in those who received on-demand treatment (n=12). In PATHFINDER-3, a clinically effective hemostatic response of "excellent" or "good" was reported with 43 out of 45 surgeries in patient ages ≥ 12 years (n=33) who received turoctocog alfa pegol as a single dose (median 51 IU/kg) prior to surgery. In PATHFINDER-5, pediatric patients 0 to 11 years of age (n=68) received prophylactic and on-demand treatment with turoctocog alfa pegol (50-75 IU/kg) twice weekly. After 26 weeks, median ABR was 1.95 episodes. Turoctocog alfa pegol was well-tolerated. An FVIII inhibitor was detected in 1 patient in PATHFINDER-1 who responded well to therapy. Hypersensitivity reactions were not reported.

PLACE IN THERAPY

Hemophilia A is a congenital X-linked bleeding disorder that affects 1 in 5,000 male births. It is characterized by coagulation FVIII deficiency leading to chronic spontaneous bleeding into muscles and joints that can progress to debilitating arthropathy. The standard of care is routine infusion of FVIII replacement products. Extended half-life FVIII products were designed to decrease the frequency of infusions. While longer half-lives have been achieved with Fc fusion (Eloctate®) and pegylation (Adynovate®, Jivi®), the actual amount of prolongation has been modest. Further, while half-life of the product may be extended in adults, it is less certain in children. Individual patient response and pharmacokinetic characteristics should be taken into account to determine optimal dosing frequency of any product.

If approved, turoctocog alfa pegol will provide another pegylated, longer-acting option for the prophylactic and on-demand treatment of hemophilia A, particularly in patients < 12 years of age. In adults, turoctocog alfa pegol's half-life is similar to Jivi and longer than Adynovate (~18.4, ~19, and ~14.7 hours, respectively). This translates to adult dosing frequency of every 4 days with turoctocog alfa pegol, twice weekly to every 5 days with Jivi, and twice weekly with Adynovate. This is compared to 3 to 4 times per week dosing with shorter half-life products. Jivi is not approved for use in pediatrics < 12 years of age; in clinical studies, adverse reactions due to immune responses to polyethylene glycol (PEG) and loss of drug effect due to neutralizing anti-PEG antibodies were reported in patients < 6 years old. Comparable safety and efficacy were demonstrated between adults and pediatrics (including < 12 years of age) for Adynovate and turoctocog alfa pegol. The Fc fusion product, Eloctate, is indicated for use in patients as young as 1 year. With an adult half-life of ~19.7 hours, Eloctate is administered IV every 4 days.

While patient convenience is an important consideration, other product nuances should be considered when prescribing FVIII replacement products. These include product purity (absence of animal-derived proteins), inhibitor development, patient pharmacokinetics, and cost. Another advancement in the hemophilia space is the monoclonal antibody emicizumab-kxwh (Hemlibra®), which is indicated for prophylaxis in hemophilia A patients with or without inhibitors. Emicizumab-kxwh provides SC dosing regimens of once every 1, 2, or 4 weeks. Gene therapy, with near elimination of bleeds, and RNA interference products are also on the horizon.

FDA APPROVAL TIMELINE

February 27, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$23	$51	$135	$163	$191

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 to be 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 market place.

In July 2018, the FDA unveiled its Biosimilar Action Plan (BAP), a series of 11 steps to encourage biosimilar market competition, some of which were previously announced or underway. BAP contains 4 key strategies: (1) improving biosimilar development and approval process; (2) maximizing scientific and regulatory clarity for sponsors; (3) effective communications for patients, clinicians, and payers; and 4) reducing unfair tactics that may delay market approval and entry. The BAP strives to promote access to biosimilar products and reduce healthcare costs.

To date, a total of 17 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)

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

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

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

While < 2% of Americans use biologics, they account for almost 40% of all prescription drug spending. Moreover, they comprised 70% of growth in drug spending from 2010 to 2015. Not surprisingly, there is a growing body of evidence on predicted biologic spend and potential biosimilar savings. The global biologic market is projected to exceed $390 billion by 2020. The global biosimilar market is expected to grow from $5.95 billion in 2018 to $23.63 billion in 2023. An IMS Health analysis expects biosimilars to save the US and Europe's top 5 markets up to $110 billion by 2020. It is estimated that in the US, biosimilars will cost 15% to 35% less than the originator product. The potential cost savings, however, can vary based on the market segment where brand contracts can play a role. A 2017 report by the RAND Corporation estimates a $54 billion cost savings from biosimilars between 2017 and 2026. In July 2018, an FDA analysis reported that if Americans had access to FDA-approved biosimilars in 2017, it

would have resulted in $4.5 billion savings. A 2017 analysis by the Moran Company projects biosimilars can save the government an estimated $11.4 billion by 2027, but it would require the CMS to revise its reimbursement policy for biosimilars. Subsequently, in November 2017, CMS revised its reimbursement policy. The CMS has begun to issue a unique Healthcare Common Procedure Coding System (HCPCS) code to each individual biosimilar. Under this new rule, Medicare Part B will separately code and pay for biosimilars and no longer group them into a common payment code with originator agents. A June 2018 study by the Pacific Research Institute, forecasts annual savings of up to $465 million from increased use of biosimilars to replace a single biologic, for commercial payers and Medicare, based on an infliximab case study.

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

Blood Modifier

adalimumab (SB5) *SC*

Samsung Bioepis/Merck

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

FDA APPROVAL TIMELINE

May 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$15,016	$15,982	$16,761	$17,136	$14,126

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 indicate 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
$2,612	$2,264	$1,922	$1,588	$1,327

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
$185	$167	$153	$141	$137

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
$ 3,055	$2,532	$2,074	$1,754	$1,485

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

Diabetes

insulin glargine (Lusduna Nexvue) *SC*

Merck

Lusduna Nexvue is a follow-on insulin to Sanofi's Lantus, a long-acting insulin indicated for the treatment of type 1 diabetes mellitus (T1DM) and type 2 diabetes mellitus (T2DM).

FDA APPROVAL TIMELINE

Pending

- Lusduna Nexvue has met all required regulatory standards for follow-on insulins regarding clinical and nonclinical safety, efficacy, and quality, but litigation claiming patent infringement invoked an automatic stay on final FDA approval for up to 30 months or a court decision in favor of Merck, whichever comes sooner.

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$1,336	$951	$685	$564	$477

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

Blood Modifier

pegfilgrastim (Lapelga) *SC*

Apotex

Lapelga is a biosimilar 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

Pending

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$3,176	$2,690	$2,296	$1,926	$1,503

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
Q3, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$3,436	$2,670	$2,158	$1,764	$1,473

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 10, 2019

FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$633	$509	$394	$320	$267

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

Oncology

trastuzumab (PF-05280014) *IV*

Pfizer

PF-05280014 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

Q1, 2019

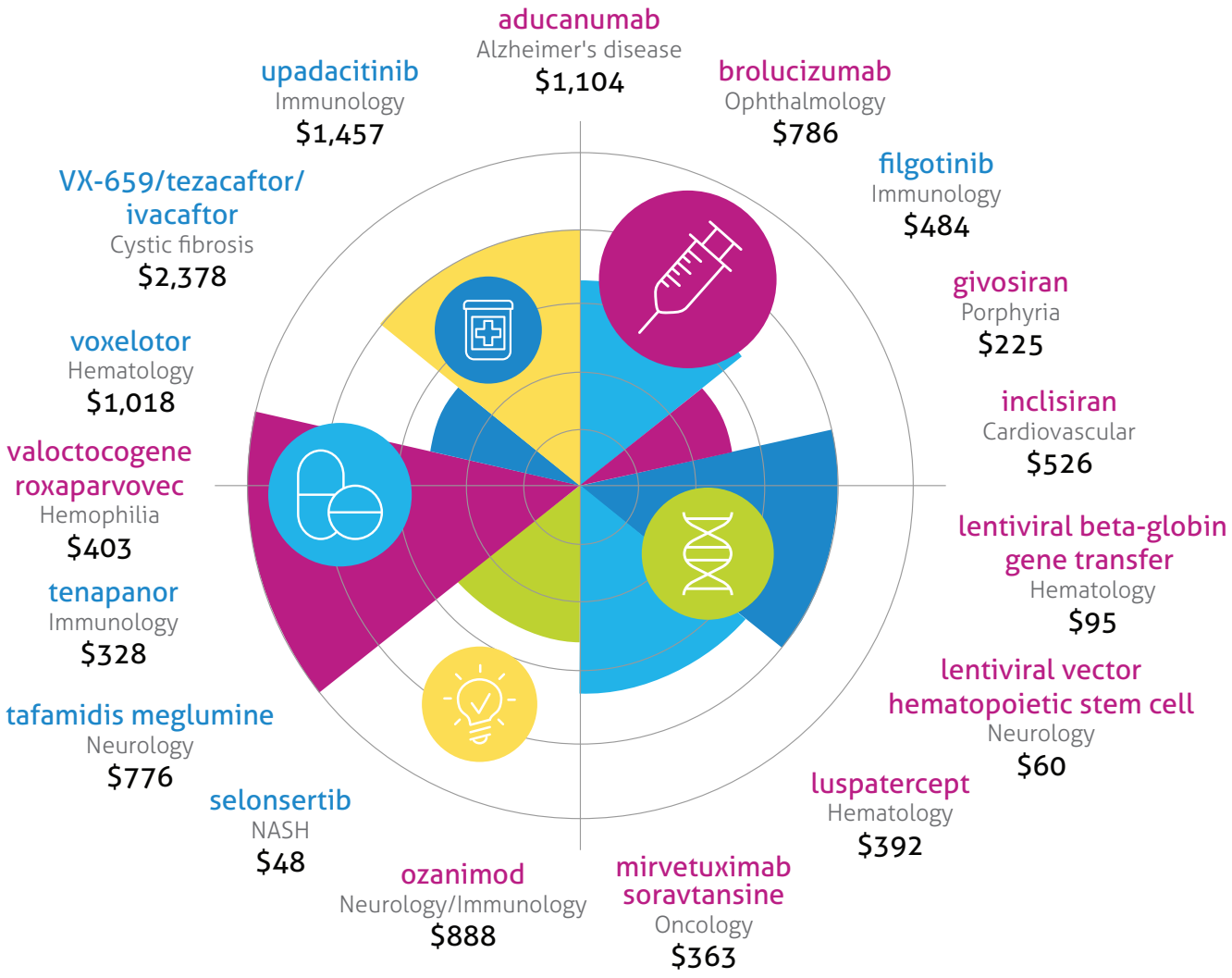
FINANCIAL FORECAST (reported in millions)

2019	2020	2021	2022	2023
$2,189	$1,729	$1,374	$1,107	$914

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.*

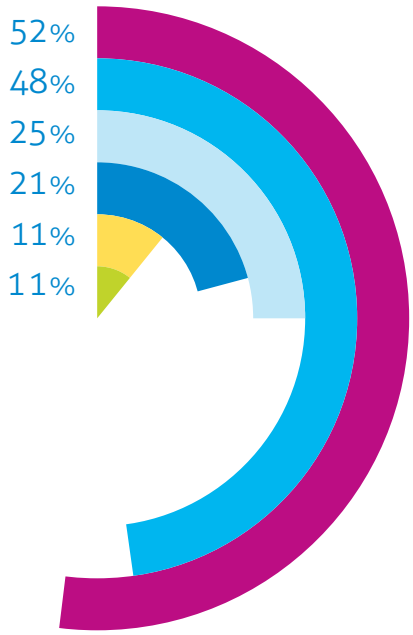


★ Specialty drug names appear in magenta throughout the publication.

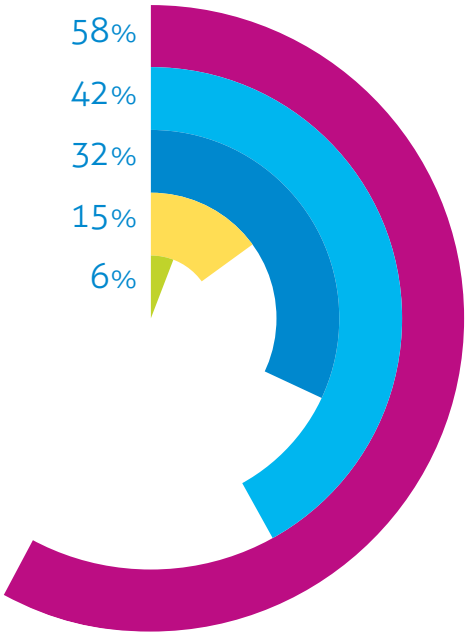
Pipeline Drug List

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	Ophthalmological indications	SC	Submitted - 505(b)(2) NDA; Fast Track	2019
levetiracetam ER	Sun	Partial seizures	Oral	Submitted - 505(b)(2) NDA	January 2019
trastuzumab (biosimilar to Genentech's Herceptin)	Pfizer	Breast cancer	IV	Submitted - BLA	Q1, 2019
apomorphine film	Sumitomo Dainippon	Parkinson's disease	SL	Submitted - 505(b)(2) NDA; Fast Track	01/29/2019
cladribine	Merck	MS (relapsing)	Oral	Submitted - NDA; Fast Track	01/30/2019
samidorphan/ buprenorphine	Alkermes	MDD	SL	Submitted - NDA; Fast Track; Priority Review	01/31/2019
insulin degludec/ liraglutide (Xultophy®)	Novo Nordisk	T2DM	SC	Submitted - sNDA	February 2019
aflibercept (Eylea®)	Regeneron	Diabetic retinopathy	Intraocular	Submitted - sBLA	Feb-Apr 2019
ruxolitinib (Jakafi®)	Incyte	Graft versus host disease (inadequate response to corticosteroids)	Oral	Submitted - sNDA; Breakthrough Therapy; Orphan Drug; Priority Review	Feb-Mar 2019
ramucirumab (Cyramza®)	Eli Lilly	HCC	IV	Submitted - sBLA; Orphan Drug	Feb-May 2019
caplacizumab	Sanofi	Thrombotic thrombocytopenic purpura (TTP)	IV	Submitted - BLA; Fast Track; Orphan Drug; Priority Review	02/06/2019
iclaprim	Motif Bio	ABSSSI	IV	Submitted - NDA; Fast Track; Priority Review; Qualified Infectious Disease Product	02/13/2019
halobetasol propionate/ tazarotene	Bausch Health	PSO	Topical	Submitted - NDA	02/15/2019
pembrolizumab (Keytruda®)	Merck	Melanoma (adjuvant, resected, high-risk stage 3)	IV	Submitted - sBLA; Breakthrough Therapy; Orphan Drug	02/15/2019
trifluridine/tipiracil (Lonsurf®)	Otsuka	Gastric cancer (advanced or metastatic)	Oral	Submitted - sNDA; Priority Review	02/24/2019
loteprednol etabonate 0.38% (sub-micron gel)	Bausch & Lomb	Ocular pain/inflammation (post-operative)	Topical	Submitted - 505(b)(2) NDA	02/25/2019
turoctocog alfa pegol	Novo Nordisk	Hemophilia A	IV	Submitted - BLA	02/27/2019
midazolam	UCB	Seizure disorders (acute treatment)	Intranasal	Submitted - 505(b)(2) NDA; Fast Track; Orphan Drug	March 2019
siponimod	Novartis	MS (secondary progressive)	Oral	Submitted - NDA; Priority Review	March 2019
dupilumab (Dupixent)	Regeneron	Atopic dermatitis (ages 12-17 years)	SC	Submitted - sBLA; Priority Review	03/11/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
atezolizumab (Tecentriq®)	Roche	Breast cancer (1st-line, unresectable advanced or metastatic, triple-negative, PD-L1+)	IV	Submitted - sBLA; Priority Review	03/12/2019
netarsudil/latanoprost	Aerie	Glaucoma/ocular hypertension	Topical	Submitted - 505(b)(2) NDA	03/14/2019
atezolizumab (Tecentriq)	Roche	Small cell lung cancer (1st-line, extensive-stage, in combination with chemotherapy)	IV	Submitted - sBLA; Orphan Drug; Priority Review	03/18/2019
brexanolone	Sage	Postpartum depression	IV	Submitted - NDA; Breakthrough Therapy; Priority Review	03/19/2019
solriamfetol	Jazz	Narcolepsy; Sleep apnea	Oral	Submitted - NDA; Orphan Drug	03/20/2019
olopatadine/mometasone furoate	Glenmark	Allergic rhinitis	Intranasal	Submitted - 505(b)(2) NDA	03/22/2019
sotagliflozin	Sanofi	T1DM	Oral	Submitted - NDA	03/22/2019
meloxicam	Recro	Postsurgical pain	IV	Submitted - 505(b)(2) NDA	03/24/2019
diazepam	Neurelis	Repetitive seizured (cluster/acute; ages ≥ 6 years)	Intranasal	Submitted - 505(b)(2) NDA; Fast Track; Orphan Drug	03/25/2019
aclidinium (Tudorza® Pressair®)	AstraZeneca	CV safety in COPD	Inhaled	Submitted - sNDA	03/31/2019
aclidinium/formoterol	Circassia	COPD	Inhaled	Submitted - NDA	03/31/2019
bevacizumab (biosimilar to Genentech's Avastin)	Pfizer	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Submitted - BLA	Q2, 2019
glucagon	Eli Lilly	Hyperinsulinemia/ hypoglycemia	Intranasal	Submitted - NDA	Apr-Jul 2019
mitomycin	Urogen	Bladder cancer	Intravesical	Submitted - 505(b)(2) NDA; Breakthrough Therapy; Orphan Drug	Apr-Sep 2019
plasminogen (human)	Prometic Life Sciences	Hypoplasminogenemia	IV	Submitted - BLA; Fast Track; Orphan Drug; Priority Review	Apr-Sep 2019
metoclopramide	Evoke	Diabetic gastroparesis	Intranasal	Submitted - 505(b)(2) NDA	04/01/2019
rizatriptan film	Intelgenx	Migraine treatment	Oral transmucosal	Submitted - 505(b)(2) NDA	04/01/2019
selinexor	Karyopharm	Multiple myeloma (penta-refractory)	Oral	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	04/05/2019
pembrolizumab (Keytruda)	Merck	NSCLC (1st-line, monotherapy, EGFR and ALK negative)	IV	Submitted - sBLA; Priority Review	04/11/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
dolutegravir/lamivudine	GlaxoSmithKline	HIV-1 infection	Oral	Submitted - NDA; Priority Review	04/18/2019
risankizumab	Abbvie	PSO	SC	Submitted - BLA	04/25/2019
alirocumab (Praluent®)	Regeneron	Major CV event reduction	SC	Submitted - sBLA	04/26/2019
bupivacaine/meloxicam	Heron	Postsurgical pain	Instillation	Submitted - NDA; Breakthrough Therapy; 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 type 1	IV	Submitted - BLA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	May 2019
trastuzumab (Herceptin)	Roche	Breast cancer	SC	Submitted - BLA	May 2019
cariprazine (Vraylar®)	Allergan	Bipolar depression	Oral	Submitted - sNDA	May-Jun 2019
esketamine	Janssen	Treatment-resistant depression	Intranasal	Submitted - NDA; Breakthrough Therapy; Fast Track	05/03/2019
amisulpride	Acacia	Post-operative nausea/ vomiting	IV	Submitted - NDA	05/05/2019
quizartinib	Daiichi Sankyo	AML (relapsed/refractory, FLT3-ITD mutated)	Oral	Submitted - NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	05/25/2019
NKTR-181	Nektar	Chronic low back pain	Oral	Submitted - NDA; Fast Track	05/28/2019
daratumumab (split-dose) (Darzalex®)	Janssen	Multiple myeloma	IV	Submitted - sBLA; Orphan Drug	06/07/2019
mannitol	Pharmaxis	CF	Inhaled	Submitted - NDA; Fast Track; Orphan Drug	06/20/2019
bremelanotide	AMAG	Hypoactive sexual desire disorder (premenopausal women)	SC	Submitted - NDA	06/21/2019
celiprolol	Acer	Ehlers-Danlos syndrome (vascular)	Oral	Submitted - NDA; Orphan Drug; Priority Review	06/25/2019
fosfomycin	Nabriva	Complicated UTI	IV	Submitted - 505(b)(2) NDA; Fast Track; Priority Review; Qualified Infectious Disease Product	06/30/2019
riluzole	Janssen	ALS	SL	Submitted - 505(b)(2) NDA; Orphan Drug	July 2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
tafamidis meglumine	Pfizer	Transthyretin amyloid (ATTR) cardiomyopathy	Oral	Submitted - NDA; Breakthrough Therapy; Fast Track; Orphan Drug; Priority Review	July 2019
ceftazidime/avibactam (Avycaz®)	Allergan	Complicated intra-abdominal infections (pediatrics); Complicated UTI (pediatrics)	IV	Submitted - sNDA; Qualified Infectious Disease Product	Jul-Aug 2019
rituximab (biosimilar to Genentech's Rituxan)	Pfizer	NHL; RA	IV	Submitted - BLA	Q3, 2019
avatrombopag (Doptelet®)	Dova	ITP	Oral	Submitted - sNDA; Orphan Drug	07/04/2019
afamelanotide	Clinuvel	Erythropoietic porphyria	Intradermal	Submitted - NDA; Fast Track; Orphan Drug; Priority Review	07/08/2019
ferric maltol	Shield	Iron deficiency anemia	Oral	Submitted - NDA	08/01/2019
filgrastim (biosimilar to Amgen's Neupogen)	Tanvex	Neutropenia/leukopenia	SC	Submitted - BLA	08/01/2019
loteprednol etabonate 0.25%	Kala	Dry eye syndrome	Topical	Submitted - 505(b)(2) NDA	08/15/2019
tasimelteon (Hetlioz®)	Vanda	Jet lag disorder	Oral	Submitted - sNDA	08/16/2019
golodirsen	Sarepta	Duchenne muscular dystrophy	IV	Submitted - NDA	08/20/2019
lefamulin	Nabriva	CAP	IV, Oral	Submitted - NDA; Fast Track; Qualified Infectious Disease Product	08/20/2019
eculizumab (Soliris®)	Alexion	Neuromyelitis optica (Devic's syndrome)	IV	Submitted - sBLA; Orphan Drug	Sep-Dec 2019
AR101	Aimmune	Peanut allergy	Oral	Submitted - BLA; Breakthrough Therapy; Fast Track	08/21/2019
atezolizumab (Tecentriq)	Roche	NSCLC (1st-line, metastatic non-squamous, EGFR-negative, ALK-negative, in combination with paclitaxel and carboplatin)	IV	Submitted - sBLA	09/02/2019
tenapanor	Ardelyx	IBS	Oral	Submitted - NDA	09/13/2019
erdafitinib	Janssen	Bladder cancer	Oral	Submitted - NDA; Breakthrough Therapy	09/18/2019
doravirine (Pifeltro™)	Merck	HIV-1 infection (antiretroviral-experienced, in combination with other antiretrovirals)	Oral	Submitted - sNDA	09/20/2019
doravirine/lamivudine/ tenofovir disoproxil fumarate (Delstrigo™)	Merck	HIV-1 infection (antiretroviral-experienced)	Oral	Submitted - sNDA	09/20/2019

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
lumateperone	Intra-Cellular Therapies	Schizophrenia	Oral	Submitted - NDA; Fast Track	09/27/2019
ivosidenib (Tibsovo®)	Agios	AML (1st-line)	Oral	Submitted - sBLA; Fast Track; Orphan Drug	October 2019
lenalidomide (Revlimid)	Celgene	Indolent lymphoma (relapsed/refractory; in combination with rituximab)	Oral	Submitted - sBLA; Orphan Drug	October 2019
deflazacort (Emflaza®)	PTC Therapeutics	Duchenne muscular dystrophy (ages 2-5 years)	Oral	Submitted - sNDA; Orphan Drug	Oct-Nov 2019
teriparatide recombinant human (follow-on to Eli Lilly's Forteo)	Pfenex	Osteoporosis	SC	Submitted - 505(b)(2) NDA	10/10/2019
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
romiplostim (Nplate®)	Amgen	ITP (resistant)	SC	Submitted - sBLA	10/18/2019
triamcinolone acetonide	Clearside Biomedical	Macular edema associated with uveitis	Intraocular	Submitted - NDA	10/18/2019
ustekinumab (Stelara®)	Janssen	UC	IV, SC	Submitted - sBLA; Orphan Drug	10/18/2019
minocycline	Foamix	Acne (ages ≥ 9 years)	Topical	Submitted - 505(b)(2) NDA	10/21/2019
tadalafil	Adamis	Erectile dysfunction	SL	Submitted - 505(b)(2) NDA	10/28/2019
naloxone	Adamis	Substance use disorder	IM	Submitted - 505(b)(2) NDA	10/31/2019
methotrexate	Cumberland	PSO	SC	Submitted - NDA	November 2019
tafamidis free acid	Pfizer	Transthyretin amyloid (ATTR) cardiomyopathy (ATTR-CM, wild type)	Oral	Submitted - NDA	November 2019
dexmethasone (Dextenza®)	Ocular Therapeutix	Ocular inflammation (post-surgical)	Intraocular	Submitted - sNDA	11/10/2019
cenobamate	SK Biopharmaceuticals	Seizure disorders	Oral	Submitted - NDA	11/26/2019
upadacitinib	Abbvie	RA	Oral	Submitted - NDA	12/20/2019
lemborexant	Eisai	Insomnia	Oral	Submitted - NDA	01/15/2020
filgrastim (biosimilar to Amgen's Neupogen)	Adello	Neutropenia/leukopenia	IV, SC	Submitted - BLA	Pending
filgrastim (biosimilar to Amgen's Neupogen)	Apotex	Neutropenia/leukopenia	IV, SC	Submitted - BLA	Pending
insulin glargine (follow-on to Sanofi's Lantus)	Merck	T1DM; T2DM	SC	Submitted - 505(b)(2) NDA	Pending

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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)	Mylan/Biocon	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)	Pfizer	RA; AS; PSO; PsA; JIA; CD; UC	SC	Phase 3 - BLA	TBD
aducanumab	Biogen	Alzheimer's disease	IV	Phase 3 - BLA; Fast Track	TBD
albuterol (ProAir RespiClick®)	Teva	COPD	Inhaled	Phase 3 - sNDA	TBD
aldoxorubicin	Nantworks	Sarcoma	IV	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
alferminogene tadenovec	Gene Biotherapeutics	Angina	Intracoronary infusion	Phase 3 - BLA; Fast Track	TBD
alicaforsen	Atlantic	UC	Rectal	Phase 3 - NDA; Fast Track; Orphan Drug	TBD
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 liposomal (Arikayce®)	Insmed	CF	Inhaled	Phase 3 - sNDA; Orphan Drug	TBD
amilomotide	Novartis	Alzheimer's disease	IM, SC	Phase 3 - BLA	TBD
andolast	Mylan	Asthma	Inhaled	Phase 3 - NDA	TBD
anifrolumab	AstraZeneca	SLE	IV	Phase 3 - BLA; Fast Track	TBD
anti-digoxin antibody	AMAG	Eclampsia/pre-eclampsia	IV	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
atezolizumab (Tecentriq)	Roche	RCC	IV	Phase 3 - sBLA	TBD
avacopan	Chemocentryx	Antineutrophil cytoplasmic antibodies (ANCA) associated vasculitis	Oral	Phase 3 - NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
avapritinib	Blueprint	Gastrointestinal stromal tumor	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
baclofen/naltrexone/ sorbitol	Pharnext	Charcot-Marie-Tooth disease	Oral	Phase 3 - NDA; Orphan Drug	TBD
baricitinib (Olumiant®)	Eli Lilly	Atopic dermatitis	Oral	Phase 3 - sNDA	TBD
bempedoic acid	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
bempedoic acid/ezetimibe	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
benralizumab (Fasenra®)	AstraZeneca	Nasal polyposis	SC	Phase 3 - sBLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Boehringer Ingelheim	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Kyowa Hakko Kirin	CRC; Glioblastoma; NSCLC; Ovarian cancer; RCC	IV	Phase 3 - BLA	TBD
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	Wet AMD	Intraocular	Phase 3 - BLA	TBD
budesonide/formoterol fumarate/glycopyrronium	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
budesonide/formoterol MDI	AstraZeneca	COPD	Inhaled	Phase 3 - NDA	TBD
C1-esterase inhibitor (Cinryze®)	Shire	Hereditary angioedema	SC	Phase 3 - sBLA	TBD
cabotegravir	GlaxoSmithKline	HIV-1 infection	IM	Phase 3 - NDA	TBD
calcipotriene/ betamethasone dipropionate	MC2 Therapeutics	PSO	Topical	Phase 3 - 505(b)(2) NDA	TBD
canagliflozin (Invokana®)	Janssen	Diabetic nephropathy	Oral	Phase 3 - sNDA	TBD
cannabidiol (Epidiolex®)	GW	Tuberous sclerosis complex	Oral	Phase 3 - sNDA; Orphan Drug	TBD
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
ceftolozane/tazobactam (Zerbaxa®)	Merck	HAP	IV	Phase 3 - sNDA; Fast Track; Qualified Infectious Disease Product	TBD
cenicriviroc mesylate	Allergan	NASH	Oral	Phase 3 - NDA; Fast Track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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
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; Breakthrough Therapy	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	Oral	Phase 3 - sNDA; Qualified Infectious Disease Product	TBD
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; Peripheral arterial disease	IM	Phase 3 - BLA; Regenerative Medicine Advanced Therapy	TBD
dupilumab (Dupixent)	Regeneron	Nasal polyposis	SC	Phase 3 - sBLA	TBD
durvalumab (Imfinzi®)	AstraZeneca	SCLC	IV	Phase 3 -sBLA	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 - NDA; Fast Track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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
eptinezumab	Alder Bio	Migraine prevention	IV	Phase 3 - BLA	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 - NDA	TBD
fedratinib	Celgene	Myelofibrosis	Oral	Phase 3 - NDA	TBD
fenfluramine	Zogenix	Dravet syndrome	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
ferric maltol	Shield	Anemia due to CKD (dialysis-independent)	Oral	Phase 3 - NDA	TBD
ferric pyrophosphate (Triferic®)	Rockwell 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 (biosimilar to EMD Serono's Gonal-F®)	Allergan	Reproductive disorder	SC	Phase 3 - BLA	TBD
formoterol fumarate MDI	AstraZeneca	COPD	Inhaled	Phase 3 - 505(b)(2) NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
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; Qualified Infectious Disease Product	TBD
galcanezumab-gnlm (Emgality®)	Eli Lilly	Cluster headache (episodic)	SC	Phase 3 - sBLA; Breakthrough Therapy; Fast Track	TBD
gefapixant	Merck	Chronic cough	Oral	Phase 3 - NDA	TBD
givosiran	Alnylam	Porphyria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
glucagon pump	Xeris	Hyperinsulinemia/ hypoglycemia	SC	Phase 3 - NDA; Orphan Drug	TBD
glycopyrrolate HFA	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 Biologics	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
ibrexafungerp	Scynexis	Fungal infections (systemic and non-systemic)	IV, Oral	Phase 3 - NDA; Fast Track; Orphan Drug; Qualified Infectious Disease Product	TBD
ibritumomab tiuxetan (Zevalin®)	Spectrum	DLBCL	IV	Phase 3 - sBLA	TBD
icosapent ethyl (Vascepa®)	Amarin	Major CV event risk reduction	Oral	Phase 3 - sNDA	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
indacaterol/ mometasone furoate/ glycopyrronium bromide	Novartis	Asthma	Inhaled	Phase 3 - NDA	TBD
inebilizumab	Viela Bio	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	T1DM; T2DM	SC	Phase 3 - 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
istradefylline	Kyowa Hakko Kirin	Parkinson's disease	Oral	Phase 3 - NDA	TBD
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis	SC	Phase 3 - sBLA	TBD
lasmiditan	Eli Lilly	Cluster headache (prevention)	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
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
lumasiran	Alnylam	Hyperoxaluria	SC	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
lumateperone	Intra-cellular Therapies	Bipolar disorder	Oral	Phase 3 - NDA	TBD
lumateperone	Ironwood	Gastroesophageal reflux disease	Oral	Phase 3 - 505(b)(2) NDA	TBD
luspatercept	Acceleron	Myelodysplastic syndrome; Thalassemia	SC	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
maribavir	Shire	Cytomegalovirus infection treatment	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
masitinib mesylate	AB Science	Asthma; Alzheimer's disease	Oral	Phase 3 - NDA	TBD
mavacamten	Myokardia	Obstructive hypertrophic cardiomyopathy	Oral	Phase 3 - NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
mepolizumab (Nucala®)	GlaxoSmithKline	Nasal polyposis	SC	Phase 3 - sBLA	TBD
meropenem/vaborbactam (Vabomere®)	Melinta	HAP; Septicemia	IV	Phase 3 - sNDA; Qualified Infectious Disease Product	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
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
nalbuphine ER	Trevi	Pruritus	Oral	Phase 3 - NDA	TBD
nitric oxide	Bellerophon	PAH	Inhaled	Phase 3 - NDA; Orphan Drug	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 - NDA	TBD
nitric oxide	AIT Therapeutics	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
olipudase alfa	Sanofi	Niemann-Pick disease	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
omalizumab (Xolair®)	Roche	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
osilodrostat	Novartis	Cushing's syndrome	Oral	Phase 3 - NDA; Orphan Drug	TBD
ozanimod	Celgene	MS; CD; UC	Oral	Phase 3 - NDA	TBD
paclitaxel (micellar)	Oasmia	Ovarian cancer	IV	Phase 3 - NDA; Orphan Drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
palovarotene	Clementia	Fibrodysplasia ossificans progressiva	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
pegunigalsidase alfa	Chiesi Farmaceutici	Fabry disease	IV	Phase 3 - BLA; Fast Track	TBD
pexidartinib	Daiichi Sankyo	Pigmented villonodular synovitis	Oral	Phase 3 - NDA; Breakthrough Therapy; Orphan Drug	TBD
pimodivir	Janssen	Influenza treatment	Oral	Phase 3 - NDA; Fast Track	TBD
pitolisant	Harmony	Narcolepsy	Oral	Phase 3 - NDA; Breakthrough Therapy; Fast Track; Orphan Drug	TBD
plasminogen (human)	Kedrion	Ligneous conjunctivitis	Ophthalmic	Phase 3 - BLA; Orphan Drug	TBD
plinabulin	Beyondspring	NSCLC; Neutropenia/ leukopenia	IV	Phase 3 - NDA	TBD
polatuzumab vedotin	Roche	DLBCL	IV	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	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
QPI-1007	Quark	Optic neuritis	Intraocular	Phase 3 - NDA; Orphan Drug	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	Graft versus host disease (GVHD)	IV	Phase 3 - BLA; Fast Track; Orphan Drug	TBD
reproxalap	Aldeyra	Congenital ichthyosis	Topical	Phase 3 - NDA; Orphan Drug	TBD
rifabutin/amoxicillin/ pantoprazole	Redhill	*H. pylori* infection	Oral	Phase 3 - NDA; Fast Track; Qualified Infectious Disease Product	TBD
rilpivirine (long-acting)	Janssen	HIV-1 infection	IM	Phase 3 - NDA	TBD
rimegepant sulfate	Biohaven	Migraine treatment	Oral	Phase 3 - NDA	TBD
risankizumab	Abbvie	CD	SC	Phase 3 - BLA; Orphan Drug	TBD
risperidone	Braeburn	Schizophrenia	SC Implant	Phase 3 - 505(b)(2) NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
rituximab (biosimilar to Genentech's Rituxan)	Archigen Biotech	NHL; RA	IV	Phase 3 - BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Amgen	NHL; RA	IV	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
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
seladelpar	Cymabay	Primary biliary cholangitis/hepatic fibrosis	Oral	Phase 3 - NDA; Orphan Drug	TBD
selinexor	Karyopharm	Sarcoma	Oral	Phase 3 - NDA; Orphan Drug	TBD
selonsertib	Gilead	NASH	Oral	Phase 3 - NDA	TBD
semaglutide (Ozempic®)	Novo Nordisk	T2DM	Oral	Phase 3 - NDA	TBD
semaglutide (Ozempic)	Novo Nordisk	Obesity	SC	Phase 3 - sNDA	TBD
serlopitant	Menlo	Pruritus associated with prurigo nodularis (PN)	Oral	Phase 3 - NDA; Breakthrough Therapy	TBD
setmelanotide	Rhythm	Obesity	SC	Phase 3 - BLA; Breakthrough Therapy; Orphan Drug	TBD
sodium oxybate (once-nightly dosing)	Avadel	Narcolepsy	Oral	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
sodium oxybate (low-dose)	Jazz	Narcolepsy	Oral	Phase 3 - NDA	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

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
sulopenem etzadroxil	Iterum	Uncomplicated UTI	IV, Oral	Phase 3 - NDA; Qualified Infectious Disease Product	TBD
talacotuzumab	Janssen	Prostate cancer (metastatic, castration resistant)	IV	Phase 3 - BLA	TBD
tanezumab	Pfizer	Osteoarthritis; Chronic low back pain; Cancer pain	IV	Phase 3 - BLA; Fast Track	TBD
tasimelteon (Hetlioz)	Vanda	Smith-Magenis syndrome-associated sleep disorder	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	Thyroid eye disease	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	Amgen	Asthma	SC	Phase 3 - BLA	TBD
timapiprant	Chiesi Farmaceutici	Asthma	Oral	Phase 3 - NDA	TBD
timbetasin	Regenerx Biopharmaceuticals	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 syndrome	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 growth hormone	Ascendis	Growth hormone deficiency	SC	Phase 3 - BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Tanvex Biopharma	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
treprostinil (patch pump)	Steadymed	PAH	SC	Phase 3 - 505(b)(2) NDA; Orphan Drug	TBD
triamcinolone acetonide	Clearside	Uveitis	Intraocular	Phase 3 - NDA	TBD
ublituximab	TG Therapeutics	CLL/SLL	IV	Phase 3 - BLA; Orphan Drug	TBD
ubrogepant	Allergan	Migraine treatment	Oral	Phase 3 - NDA	TBD
udenafil	Allergan	Erectile dysfunction	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	FDA APPROVAL
upadacitinib	Abbvie	Atopic dermatitis; PsA; CD; UC; Axial spondyloarthritis	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 (ages 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 syndrome	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	Hereditary transthyretin (hATTR) amyloidosis polyneuropathy; Transthyretin amyloid cardiomyopathy (ATTR-CM, wild type or hereditary)	SC	Phase 3 - NDA	TBD
VX-445/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA; Fast Track	TBD
VX-659/tezacaftor/ivacaftor	Vertex	CF	Oral	Phase 3 - NDA	TBD
zanubrutinib	Beigene	Waldenström 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
aflibercept prefilled-syrnge (Eylea®)	Regeneron	Wet AMD	Intraocular	CRL	TBD
apremilast (Otezla®) - once-daily	Celgene	PSO	Oral	Withdrawn	N/A
astodrimer sodium	Starpharma	Bacterial vaginosis (treatment & prevention)	Intravaginal	CRL	TBD
gepirone	GlaxoSmithKline	MDD	Oral	CRL	TBD
immunoglobulin (Bivigam®)	ADMA	Primary immunodeficiencies	IV	CRL	TBD
macitentan (Opsumit®)	Actelion	Pulmonary hypertension (inoperable)	Oral	CRL	TBD
mepolizumab (Nucala®)	GlaxoSmithKline	COPD	IV, SC	CRL	TBD
nivolumab (Opdivo®)	Bristol-Myers Squibb	NSCLC (1st-line with ipilimumab)	IV	Withdrawn	N/A
oliceridine	Trevena	Acute pain	IV	CRL	TBD
oxycodone (abuse-deterrent)	Mallinckrodt	Pain	Oral	CRL	TBD
pembrolizumab (Keytruda)	Merck	SCCHN (recurrent, metastatic)	IV	CRL	TBD
sacituzumab govitecan	Immunomedics	Breast cancer	IV	CRL	TBD
tadalafil	Aquestive	Erectile dysfunction	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

AMD Age-Related Macular Degeneration

AML Acute Myeloid Leukemia

ANDA Abbreviated New Drug Application

AS Ankylosing Spondylitis

BED Binge Eating Disorder

BLA Biologics License Application

BsUFA Biosimilar User Fee Act

CAP Community Acquired Pneumonia

CD Crohn's Disease

CDC Centers for Disease Control and Prevention

CF Cystic Fibrosis

CHF Congestive Heart Failure

CI Confidence Interval

CKD Chronic Kidney Disease

CLL Chronic Lymphocytic Leukemia

COPD Chronic Obstructive Pulmonary Disease

CRC Colorectal Cancer

CRL Complete Response Letter

CV Cardiovascular

CVD Cardiovascular Disease

DEA Drug Enforcement Administration

DLBCL Diffuse Large B Cell Lymphoma

DPP-4 Dipeptidyl Peptidase 4

DR Delayed-Release

EDSS Expanded Disability Status Scale

EGFR Epidermal Growth Factor Receptor

ER Extended-Release

FDA Food and Drug Administration

FLT3 FMS-Like Tyrosine Kinase-3

GI Gastrointestinal

GLP-1 Glucagon-Like peptide-1

H Half

HAP Healthcare-Associated Pneumonia

HAM-D Hamilton Depression Rating Scale

HbA1c Hemoglobin A1c

HCC Hepatocellular Carcinoma

HCP Healthcare Professional

HCV Hepatitis C Virus

HER Human Epidermal Growth Factor Receptor

HER2 Human Epidermal Growth Factor Receptor 2

HFA Hydrofluoroalkane

HIT Heparin Induced Thrombocytopenia

HIV-1 Human Immunodeficiency-1 Virus

HTN Hypertension

HR Hazard Ratio

IBS Irritable Bowel Syndrome

IM Intramuscular

IV Intravenous

JIA Juvenile Idiopathic Arthritis

LDL-C Low-Density Lipoprotein Cholesterol

MADRS Montgomery–Åsberg Depression Rating Scale

MDD Major Depressive Disorder

MDI Metered Dose Inhaler

MS Multiple Sclerosis

GLOSSARY continued

N/A Not Applicable

NASH Non-Alcoholic Steatohepatitis

NDA New Drug Application

NHL Non-Hodgkin Lymphoma

NSAID Non-Steroidal Anti-Inflammatory Drug

NSCLC Non-Small Cell Lung Cancer

OS Overall Survival

PAH Pulmonary Arterial Hypertension

PARP poly(ADP-ribose) polymerase

PASI 50 Psoriasis Area and Severity Index ≥ 50%

PASI 70 Psoriasis Area and Severity Index ≥ 70%

PASI 90 Psoriasis Area and Severity Index ≥ 90%

PCI Percutaneous Coronary Intervention

PD-1 Programmed Death Protein 1

PD-L1 Programmed Death-Ligand 1

PDUFA Prescription Drug User Fee Application

PFS Progression-Free Survival

PGA Physicians Global Assessment

PsA Psoriatic Arthritis

PSO Plaque Psoriasis

PTCA Percutaneous Transluminal Coronary Angioplasty

Q Quarter

QOL Quality of Life

RA Rheumatoid Arthritis

RCC Renal Cell Carcinoma

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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