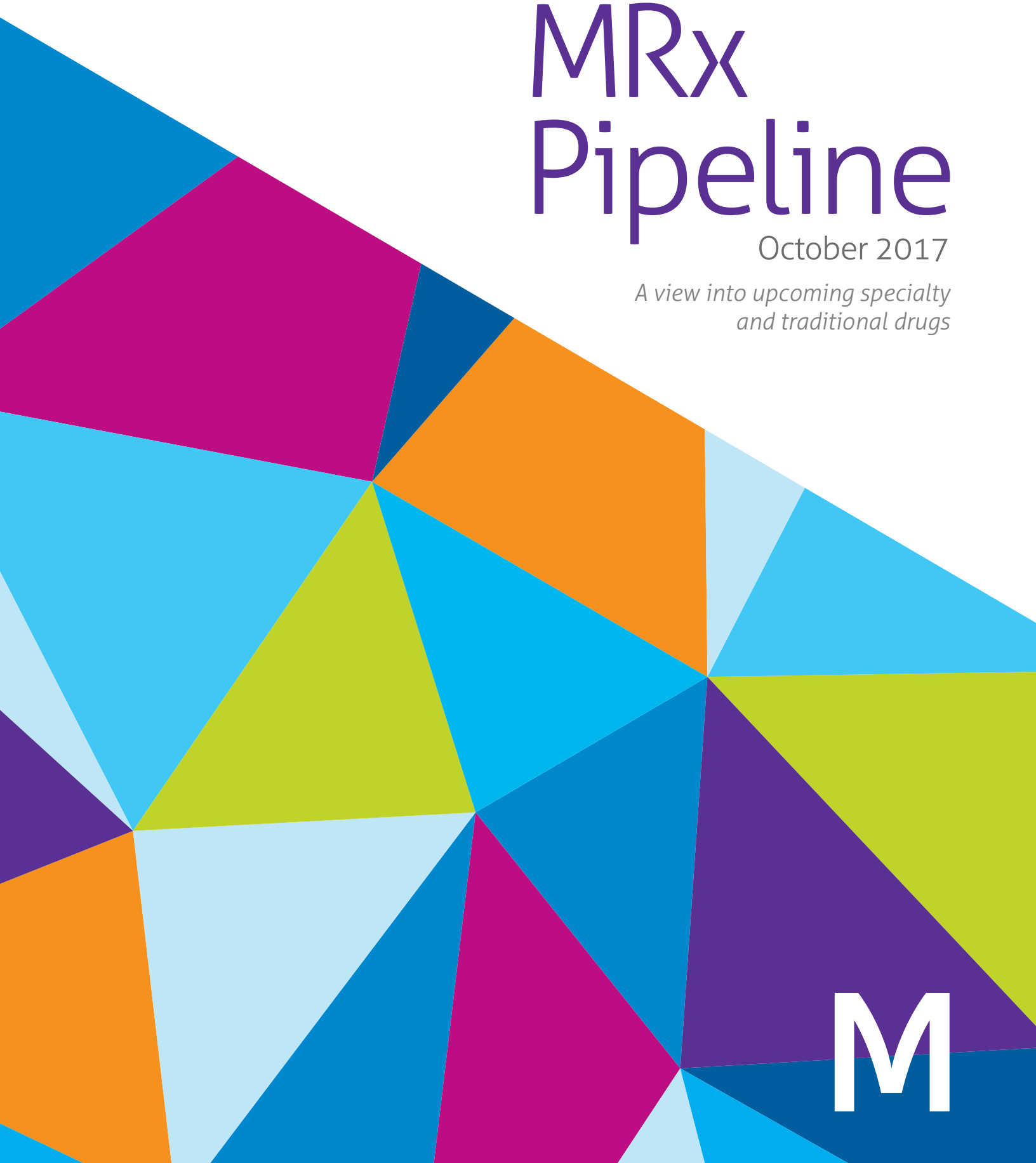
Magellan Rx MANAGEMENT℠

MRx Pipeline

October 2017

A view into upcoming specialty and traditional drugs



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INTRODUCTION

Welcome to the *MRx Pipeline*. 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 to assess the potential impact of these pipeline drugs, where available, a financial forecast has been included for select products. Complemented by data from Evaluate™, this pipeline report looks ahead at the 5-year projected total annual US sales through the year 2021. These figures are not specific to a particular commercial or government line of business, rather look at forecasted sales in the US. 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.

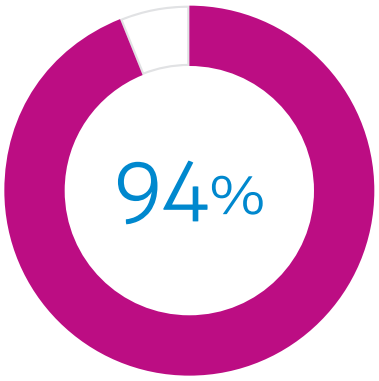
In the past few years, game changers such as products in the hepatitis C and immunotherapy fields have blazed the pipeline trail. A continued key trend toward the approval of specialty medications is expected. Noteworthy pipeline trends to watch in the upcoming quarters include the development of complex therapies, rare diseases, oncology, immunology, hemophilia, Alzheimer's disease, migraine prophylaxis, growth of biosimilars, and new treatment modalities using gene therapy.

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.

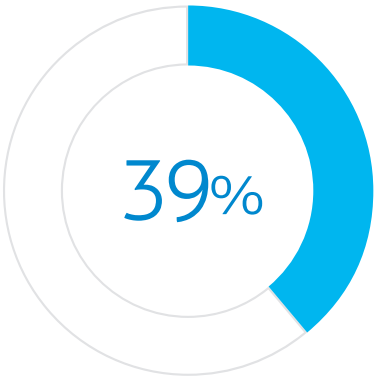
Pipeline Deep Dive

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.

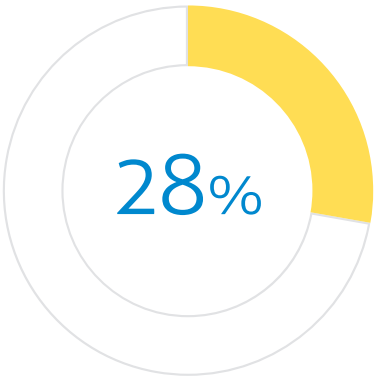
SPECIALTY



PRIORITY REVIEW

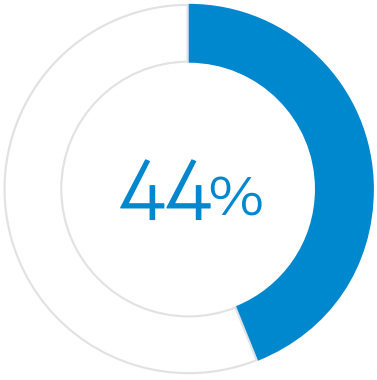


BREAKTHROUGH THERAPY



BIOSIMILAR

ORPHAN DRUG



★ Specialty drug names appear in magenta throughout the publication.

ataluren (Translarna) *oral*

PTC Therapeutics

PROPOSED INDICATIONS

Nonsense mutation Duchenne muscular dystrophy (DMD)

CLINICAL OVERVIEW

In DMD, a nonsense mutation in the dystrophin gene results in the incomplete production of dystrophin, a critical protein in muscle structure and function. DMD, is characterized by muscular degeneration with muscle weakness seen as early as 3 years of age, which progresses to respiratory and cardiac dysfunction and premature death. DMD primarily affects boys, but in rare cases, it can affect girls. Ataluren allows the DNA error to be "read through" in the translation process, resulting in a fully functional dystrophin protein.

A pivotal double-blind, phase 3 trial (ACT DMD) evaluated the efficacy of ataluren 40 mg/kg/day (given orally in 3 divided doses) versus placebo in 228 males, 7 to 16 years of age, with nonsense mutation DMD. Overall, ataluren resulted in a nonsignificant benefit in change in 6-minute walking distance (6MWD) at 48 weeks compared to placebo (-47.7 meters versus -60.7 meters, respectively). However, a significant difference (15.9 meters) in change in 6MWD and a slower rate of decline in physical function was seen between the ataluren and placebo groups in 1 prespecified subgroup of patients who had a baseline 6MWD of 300 meters to < 400 meters. Adverse effects reported more often with ataluren than placebo in the phase 3 trial included diarrhea (17%) and upper respiratory tract infections (10%). A phase 2b open-label study showed no improvement compared to placebo in 6MWD, even when a higher ataluren dose of 80 mg/kg/day (3 divided doses) was used. Although, clinical trials report ataluren as generally well tolerated, abbreviated prescribing information published by PTC advises to closely monitor patients with renal or hepatic impairment and to measure blood pressure and serum lipid levels.

PLACE IN THERAPY

Approximately 10% to 15% of DMD cases (~2,000 patients) in the US have nonsense mutations. If approved, ataluren will be the third agent approved in the US to treat DMD, following the antisense oligonucleotide, eteplirsen (Exondys 51™), and the oral glucocorticoid, deflazacort (Emflaza™). Both ataluren and eteplirsen treat the underlying disease. While IV administered eteplirsen is indicated in patients with a confirmed gene mutation amenable to exon 51 skipping, found in approximately 13% of cases, oral ataluren is seeking an indication directed toward the nonsense mutation. Notably, deflazacort does not treat the underlying cause of DMD; rather it exerts anti-inflammatory and immunosuppressive effects to delay disease progression.

Long-term safety and efficacy studies are lacking for ataluren and eteplirsen. Moreover, available data supporting effectiveness of both drugs are controversial. On September 28, 2017, the FDA's advisory committee concluded by majority vote that data provided by PTC are inconclusive to establish ataluren's effectiveness in DMD; however, no member voted that the data suggest that it is ineffective in DMD. While not mandated, FDA decisions are typically consistent with its advisory committee recommendations. The ataluren NDA was Filed Over Protest, which allows submission and review of a product after a Refuse To File FDA determination.

FDA APPROVAL TIMELINE

October 24, 2017

✓ Fast track ✓ Orphan drug

FINANCIAL FORECAST (reported in millions)

Ataluren is approved in Germany for nonsense mutation in DMD in patients ≥ 5 years of age. The German list price is approximately €440,000 per year. This equates to about $515,000 per year.

Oncology

axicabtagene ciloleucel *IV*

Gilead/Kite

PROPOSED INDICATIONS

Refractory aggressive non-Hodgkin lymphoma (NHL) in patients who are ineligible for autologous stem cell transplant (SCT)

CLINICAL OVERVIEW

Chimeric antigen receptor T cell (CAR-T) therapy is a novel immunotherapy that helps the patient's own immune system fight cancer. With CAR-T, the patient's T cells are collected from their blood, then genetically altered to produce proteins on the cell surface called chimeric antigen receptors (CARs). Once allowed to multiply *in vitro,* the re-engineered T cells are infused back into the same patient as a single dose. With axicabtagene ciloleucel, the CAR modified T cells recognize and attack cancerous B cells that express CD19 on their cell surface. Cytokine-release syndrome (CRS) and neurotoxicity are serious or life-threatening toxicities reported with CAR-T therapy. Serious cases are managed with the interleukin-6 inhibitor, tocilizumab (Actemra®), which gained FDA-approval for this indication in September 2017.

In the pivotal, phase 1/2, open-label, ZUMA-1 trial, adult patients who did not respond to chemotherapy or had relapsed within 12 months of an autologous SCT, received an IV infusion of axicabtagene ciloleucel after fludarabine/cyclophosphamide lymphodepleting chemotherapy. At 12 months, the overall response rate (ORR) was 82%, with a complete response (CR) in 54% of patients. Response was consistent across disease subtypes including diffuse large B cell lymphoma [DLBCL], primary mediastinal large B cell lymphoma (PMBCL), and transformed follicular lymphoma (TFL). The rate of grade 3 or higher CRS and neurologic events was 13% and 28%, respectively. Two reported deaths were deemed treatment-related and associated with CRS; 1 was due to hemophagocytic lymphohistiocytosis and 1 was due to cardiac arrest.

Axicabtagene ciloleucel is given as a single IV infusion.

PLACE IN THERAPY

In 2017, it is estimated that over 72,000 people will be diagnosed with NHL, most occurring after the age of 45 years. Five-year survival rate for NHL is 71%.

The majority of NHL cases (85%) are B cell type lymphomas; while some aggressive forms are often curable with intensive chemotherapy, others are less responsive. For patients who are refractory to or relapse after chemotherapy or SCT, there remain few options. Single-dose CAR-T therapies may prove to be valuable tools for salvage therapy and are expected to have less mid- to long-term toxicity compared to SCT, despite the initial acute toxicity. However, long-term studies are needed to confirm safety and durability.

Axicabtagene ciloleucel could be the second CAR-T therapy approved in the US. The first CAR-T gene therapy, tisagenlecleucel (Kymriah™), received approval for treatment of acute lymphoblastic leukemia (ALL) in August 2017. Their use will be limited to certified centers that are able to handle cryopreserved cells and are experienced in treating high-risk hematologic cancers.

FDA APPROVAL TIMELINE

November 29, 2017

✓ Breakthrough therapy ✓ Orphan drug *(Diffuse large B cell lymphoma only)* ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 2	$ 171	$ 428	$ 633	$ 866

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

Metabolic

beta-glucuronidase (recombinant, human; rhGUS) *IV*

Ultragenyx

PROPOSED INDICATIONS

Mucopolysaccharidosis VII (MPS VII)

CLINICAL OVERVIEW

rhGUS is an enzyme replacement therapy being studied for the treatment of MPS VII, also known as Sly syndrome.

MPS VII belongs to a group of autosomal recessive genetic metabolic diseases known as lysosomal storage disorders. Mutations in the GUSB gene cause a deficiency of beta-glucuronidase, an intracellular enzyme that breaks down certain carbohydrates and fats. Accumulation of complex carbohydrates (mucopolysaccharides; glycosaminoglycans [GAG]) in cells (lysosomes) within the CNS, bones, joints, and various organs results. Age of onset and disease severity vary. Death during or shortly after gestation may occur in severe cases. In milder cases, skeletal abnormalities, short stature, and some degree of intellectual disability are distinguishing features; macrocephaly, corneal opacity, hepatosplenomegaly, cardiac abnormalities, and osteoarthritis may also be seen.

A small randomized, placebo-controlled, blind-start, cross-over, phase 3 study, was conducted in 12 patients ages 5 to 35 years with MPS VII. It demonstrated a significant (64.8%) reduction from baseline in urinary GAG (uGAG) excretion after 24 weeks of rhGUS treatment. The composite of walking, pulmonary function, shoulder flexion, visual acuity, and fine and gross motor skills was assessed with the multi-domain responder index (MDRI), which revealed an overall mean domain improvement of +0.5 in patients treated with rhGUS; however, this fell short of statistical significance. Notably, numerical improvements in walking distance (20.8 ± 16.75 meters) were reported in 9 patients who experienced a change from baseline; while 3 of the patients experienced significant improvement (≥ 65 meters), the other 6 showed a slight decline. Improvements in fatigue were also reported in most patients at some point during the study. The most common adverse events were mild to moderate infusion-related reactions.

The dosage studied for rhGUS was 4 mg/kg infused IV every other week.

PLACE IN THERAPY

MPS VII is an ultra rare condition with less than 100 cases reported in the US. Limited data suggest that hematopoietic SCT may slow or prevent neurological complications. While, the small pivotal study showed a significant improvement in the biomarker uGAG with rhGUS, improvement in a composite score of physical and pulmonary function and vision narrowly missed significance. Enzyme replacement therapy has been FDA-approved for other forms of MPS; if approved, rhGUS will be the first to treat MPS VII.

FDA APPROVAL TIMELINE

November 16, 2017

✓ Fast track ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 5	$ 12	$ 15	$ 18

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

HIV/AIDS

bictegravir/ emtricitabine/ tenofovir alafenamide (BIC/FTC/TAF) *oral*

Gilead

PROPOSED INDICATIONS

Human immunodeficiency virus (HIV)-1 infection

CLINICAL OVERVIEW

Bictegravir (BIC) is a next-generation integrase inhibitor (INSTI). It does not require boosting with the addition of another drug, such as ritonavir or cobicistat, to increase drug levels in the body. It is being combined with the nucleoside analog emtricitabine (FTC) and the nucleoside reverse transcriptase inhibitor tenofovir alafenamide (TAF).

Four pivotal, 48-week, phase 3 trials evaluated safety and efficacy of coformulated BIC/FTC/TAF in HIV-positive adults. Among 2 of the trials, BIC/FTC/TAF demonstrated non-inferiority compared to abacavir/dolutegravir/lamivudine (ABC/DTG/3TC; Triumeq®) and DTG (Tivicay®) + FTC/TAF (Descovy®) in treatment-naïve patients. In the other 2 trials, BIC/FTC/TAF demonstrated non-inferiority in virologically-suppressed patients (HIV-1 RNA < 50 copies/mL) who switched from either ABC/DTG/3TC or DTG + ABC/3TC (Epzicom®) or regimens that contained a boosted protease inhibitor (darunavir or atazanavir) plus nucleoside/nucleotide backbone (ABC/3TC or FTC/tenofovir disoproxil fumarate [TDF]). The adverse effect profile was similar with BIC/FTC/TAF and the other drug regimens; however, nausea was reported more often with ABC/DTG/3TC than with BIC/FTC/TAF (23% versus 10%). A phase 2 trial compared BIC/FTC/TAF with DTG/FTC/TAF in antiretroviral-naïve patients (n=98). Both regimens resulted in similar high virological response at 24 and 48 weeks (97% with BIC/FTC/TAF at weeks 24 and 48; 94% and 91% with DTG/FTC/TAF at weeks 24 and 48, respectively). No treatment-emergent resistance was evident. A phase 2/3 study is ongoing in virologically-suppressed pediatric patients (6 to 17 years of age).

BIC/FTC/TAF was studied at a fixed-dose of 50/200/25 mg administered orally, once daily, without regard to food.

PLACE IN THERAPY

If BIC/FTC/TAF is approved, Gilead will introduce a new INSTI, bictegravir, in a fixed-dose triple combination product to compete with GlaxoSmithKline's Triumeq, which contains the INSTI dolutegravir. Both products do not require boosting and provide a single-tablet, once-daily option in the armamentarium against HIV-1 infection. Bictegravir shows promising efficacy results with a potentially high *in vitro* resistance barrier.

FDA APPROVAL TIMELINE

February 12, 2018

✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 519	$ 1,240	$ 1,855	$ 2,411

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

Hemophilia

emicizumab *sc*

Roche

PROPOSED INDICATIONS

Hemophilia A with factor VIII inhibition

CLINICAL OVERVIEW

Emicizumab is a bispecific antibody that simultaneously binds factor IXa and factor X to activate the coagulation cascade. This mechanism may allow normal clotting in patients with hemophilia A who develop inhibitors (antibodies) to standard factor VIII replacement therapies. The development of inhibitors is the most significant treatment complication in hemophilia patients. The development of inhibitors reduces the efficacy of standard therapy, the control of bleeds, and may increase patient morbidity. The presence of inhibitors in a patient may require alternative therapies to be used.

Safety and efficacy were evaluated in 2 phase 3, open-label trials in patients with hemophilia A and inhibitors to factor VIII. The HAVEN-1 trial enrolled 109 male patients ≥ 12 years of age. Prophylaxis with emicizumab demonstrated an 87% reduction in annualized bleeding rate compared to no prophylaxis (2.9 versus 23.3 events, respectively). Moreover, no bleeding events were reported in 63% of patients treated with emicizumab as compared to 6% of patients who received no prophylaxis. In patients who had received prior bypassing agent (BPA) prophylaxis and were assigned to emicizumab prophylaxis, the annualized bleeding rate was 79% lower than with previous BPA prophylaxis. Interim data from the single-arm, HAVEN-2 trial revealed similar results with emicizumab prophylaxis in 19 children < 12 years of age who required previous BPA therapy. Common adverse effects included injection-site reaction, headache, fatigue, upper respiratory tract infection, and arthralgia. No antidrug antibodies were detected.

Emicizumab study dose was 3 mg/kg SC weekly for 4 weeks, followed by 1.5 mg/kg weekly thereafter.

PLACE IN THERAPY

It is estimated that about 20,000 people in the US have hemophilia, of whom about 80% to 85% have hemophilia A. Inhibitors develop in 20% to 33% of patients diagnosed with moderately severe to severe hemophilia A, and at a much lower rate, 5% to 10%, in patients characterized as having mild to moderate hemophilia A. Inhibitors do not change the frequency or severity of bleeds, but do affect the efficacy of treatments. BPA therapy, either on-demand or as prophylaxis, circumvents the factors that are blocked by the inhibitor to help clot formation; although, treatment with BPAs is less predictable than with standard factor replacement therapy.

Currently available BPAs, Feiba® (anti-inhibitor coagulant complex; Shire) and NovoSeven® RT (recombinant factor VIIa; Novo Nordisk) are administered IV and have short durations of action. Emicizumab is anticipated to have a longer half-life, duration of action, and is administered via SC injection. Emicizumab is also being studied in patients without factor VIII inhibitors; NovoSeven RT and Feiba are not indicated in this subpopulation. Interim data are expected in late 2017.

FDA APPROVAL TIMELINE

February 23, 2018

✓ Breakthrough therapy ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 119	$ 324	$ 580	$ 785

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

Neurology

erenumab (Aimovig) *SC*

Amgen/Novartis

PROPOSED INDICATIONS

Migraine prevention

CLINICAL OVERVIEW

Calcitonin gene-related peptide (CGRP), a signaling neuropeptide released from activated trigeminal sensory afferent neurons, convey sensory stimuli to the brain during a migraine attack. Erenumab is a monoclonal antibody that inhibits the CGRP receptor.

Safety and efficacy of erenumab were evaluated in 2 pivotal, double-blind, placebo-controlled, phase 3 trials, ARISE (12-week) and STRIVE (24-week), in patients (n=1,532) who experienced 4 to 14 migraines per month (median, 8.3). Erenumab 70 mg SC once monthly resulted in a statistically significant reduction from baseline in monthly migraine days (MMD) (ARISE: -2.9 days; STRIVE: -3.2 days) compared to placebo (-1.8 days in both studies); in STRIVE, a monthly dose of 140 mg also produced a 3.7-day reduction in MMD. In addition, 43% to 50% of patients achieved ≥ 50% reduction in MMD with erenumab (70 mg and 140 mg, respectively) compared to 27% with placebo in STRIVE. A similar phase 2 trial (n=667) demonstrated a significant reduction in MMD with both doses of erenumab in patients who experienced ≥ 15 headache days per month (≥ 8 considered migraine) and failed ≥ 2 prior preventive treatments (-6.6 days for erenumab; -4.2 days for placebo). Erenumab was well tolerated; the most common adverse effects were nasopharyngitis, injection site pain, upper respiratory tract infection, and sinusitis.

PLACE IN THERAPY

Over 37 million Americans, the majority of whom are women, suffer from migraine attacks. Migraines are painful, often debilitating, and contribute to absenteeism and reduced quality of life. Studies suggest that 38% to 50% of migraineurs, who suffer severe and/or frequent attacks, are appropriate candidates for preventive therapy, but only an estimated 6% to 13% are actually on prophylaxis. Select anticonvulsants and antihypertensives, triptans (short-term for menstrual migraines), and select antidepressants are considered effective or possibly effective for migraine prevention. OnabotulinumtoxinA (Botox®) injection is indicated in patients who experience (≥ 15 MMD). Adherence to preventive therapy is low (approximately 20% at 1 year), often due to side effects and inability to fully eliminate headache pain.

Erenumab targets episodic migraine prevention using a new approach. It appears to be well tolerated and may be effective in patients who have failed prior migraine prophylaxis therapy. Several other agents targeting CGRP are also in late-stage development for migraine prevention, including atogepant (oral), and the monoclonal antibodies: eptinezumab (SC), fremanezumab (SC), and galcanezumab (SC). Submissions for FDA-approval are expected by the end of 2017 for fremanezumab and galcanezumab, and in the second half of 2018 for eptinezumab.

FDA APPROVAL TIMELINE

May 17, 2018

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 185	$ 377	$ 552	$ 725

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

HIV/AIDS

ibalizumab *IV*

Theratechnologies

PROPOSED INDICATIONS

Human immunodeficiency virus (HIV)-1 infection

CLINICAL OVERVIEW

Ibalizumab is a humanized anti-CD4 antibody that binds to the CD4 receptor and potentially prevents entry of HIV into CD4+ T cells.

Safety and efficacy of ibalizumab given with optimized background antiretroviral therapy (ART) were evaluated in a pivotal, single-arm, phase 3 trial in adults (n=40) with multi-drug resistant (MDR) HIV-1. Seven days after an ibalizumab loading dose, 83% of patients achieved at least a 5 $\log^{10}$ decrease in viral load from baseline. At 24 weeks, the mean viral load reduction was 1.6 $\log^{10}$, with over 48% of patients experiencing a reduction greater than 2 $\log^{10}$. A total of 43% of patients had undetectable viral load (HIV-1 < 50 copies/mL) and 50% had a viral load < 200 copies/mL. After 24 weeks, the study reported a mean increase in CD4+ T cell of 48 cells/µL. Most treatment-emergent adverse events were mild to moderate in severity; however, there was 1 report of immune reconstitution inflammatory syndrome, a response that may be triggered by a change to more potent ART.

Ibalizumab was given as a single loading dose of 2,000 mg IV and then 800 mg IV every 2 weeks through 24 weeks.

PLACE IN THERAPY

Approximately 1.2 million people in the US are HIV-positive, of which about 10,000 have MDR HIV.

If approved, ibalizumab may offer a new option to patients with MDR who have limited alternatives. Providing the first new mechanism of action to treat HIV-1 infection in nearly a decade, it blocks the entry of the virus into the CD4 cell, rather than inhibiting viral replication as seen with existing ART. Further, ibalizumab will be the first long-acting HIV treatment, dosed IV every 2 weeks.

FDA APPROVAL TIMELINE

January 3, 2018

✓ Breakthrough therapy ✓ Fast track ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 38	$ 77	$ 123	$ 164

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

Respiratory

tezacaftor/ ivacaftor *oral*

Vertex

PROPOSED INDICATIONS

Cystic fibrosis (CF) in patients ≥ 12 years of age with 2 copies of the *F508del* mutation or 1 copy of the *F508del* mutation and 1 residual function mutation

CLINICAL OVERVIEW

CF affects multiple organ systems in the body particularly the lungs and digestive tract. It is caused by a variety of defects in the CF gene, which codes for the CF transmembrane conductance (CFTC) protein that functions as a chloride channel. The most common defect is *F508del,* resulting in abnormal processing of the CFTC protein. Tezacaftor correctors the defect in the protein and allows it to reach the cell surface. Once at the surface, the CFTR protein potentiator, ivacaftor, enhances CFTR protein function to increase chloride transport across mucosal epithelial cells and improve lung function.

Safety and efficacy of tezacaftor/ivacaftor was evaluated in 2 phase 3, randomized, double-blind, placebo-controlled, parallel-group, 24-week trials in patients ≥ 12 years of age with either 2 copies of the *F508del* mutation (EVOLVE; n=504) or with 1 copy of the *F508del* mutation plus a second CFTR mutation predicted to have residual function (EXPAND; n=161). The mean absolute improvement in percentage of predicted forced expiratory volume in 1 second ($ppFEV_1$) with tezacaftor/ivacaftor was statistically significant in both trials compared to placebo (difference 4% in EVOLVE, 6.8% in EXPAND). A pre-specified analysis in EXPAND showed that tezacaftor/ivacaftor provided a statistically significant improvement in $ppFEV_1$ over ivacaftor alone (2.1%). Tezacaftor/ivacaftor was generally well tolerated, with a similar occurrence of adverse effects to placebo.

The study regimen was administered orally as 1 fixed-dose tablet of tezacaftor/ivacaftor (100 mg/150 mg) in the morning and 1 ivacaftor 150 mg tablet in the evening.

PLACE IN THERAPY

CF is diagnosed in approximately 30,000 people in the US. The median survival is 36.9 years; although with current treatments, children with CF are expected to live approximately 40 years.

The *F508del* mutation is the most common mutation associated with CF. Approximately 45% of CF patients in the US have 2 copies of the *F508del* mutation. Over 900 patients with CF have 1 of 23 residual function mutations that results in partially functioning ("residual functioning") CFTR protein. Tezacaftor/ivacaftor will be the second product in the US to combine a CFTR potentiator and CFTR corrector to treat CF due to *F508del* mutation. The first, Vertex's lumacaftor/ivacaftor (Orkambi®), is indicated in patients age ≥ 6 years with 2 copies of the *F508del* mutation, but is not indicated in those with residual function mutation. Ivacaftor alone (Kalydeco®), also by Vertex, is approved in patients ≥ 2 years of age with 1 CFTR gene mutation that is responsive to ivacaftor.

FDA APPROVAL TIMELINE

February 28, 2018

✓ Breakthrough therapy ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 140	$ 372	$ 554	$ 528

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

Ophthalmology

voretigene neparvovec (Luxturna) *intraocular*

Spark

PROPOSED INDICATIONS

Biallelic *RPE65*-mediated inherited retinal disease (IRD)

CLINICAL OVERVIEW

Mutations in the *RPE65* gene are associated with progressive loss of vision, ultimately leading to blindness. Voretigene neparvovec is an adeno-associated virus (AAV) derived gene therapy vector that delivers a functional copy of the *RPE65* gene to the eye, essential for the regeneration of the visual pigment necessary for vision.

Safety and efficacy of voretigene neparvovec were assessed in an open-label, phase 3 trial in eligible patients aged ≥ 3 years with confirmed genetic biallelic *RPE65* mutations. A statistically significant and clinically meaningful improvement in visual mobility, as detected by the change in mean bilateral multi-luminance mobility testing (MLMT) that evaluates the ability to navigate a mobility course under a variety of light intensity levels, was achieved in patients treated with voretigene neparvovec (n=21) compared to those in the control group (n=10) (difference of 1.6; 95% CI, 0.72, 2.41). Improved vision was detected 30 days after dose administration and was maintained at the 1-year study endpoint. While the study demonstrated full-field light sensitivity threshold testing for white light and mobility testing with voretigene neparvovec, it did not meet statistical significance in visual acuity. In addition, in phase 1 studies, durability of response has been demonstrated for up to 4 years. The most common ocular adverse events were mild and included transient ocular inflammation, transient elevated intraocular pressure, cataracts, and intraoperative retinal tears.

Voretigene neparvovec was studied at a single dose of 1.5×10^{11} vector genomes in 0.3 mL, administered via subretinal injection.

PLACE IN THERAPY

IRDs are estimated to affect approximately 200,000 people in the US. Preliminary estimates suggest that 1,100 to 3,300 individuals may have *RPE65*-mediated IRD. Over 60 different mutations in the *RPE65* gene alone have been associated with IRDs. The most severe IRD due to *RPE65* mutation is Leber's congenital amaurosis, in which blindness may be present at birth or early childhood.

Gene therapy is currently on the forefront of drug research for the treatment of many disorders including rare diseases. AAV vectors are a promising vehicle for therapeutic gene delivery to the retina. While the damage in IRD is irreversible, voretigene neparvovec may be the first treatment option to improve functional vision, light sensitivity, and visual field for patients with biallelic *RPE65*-mediated IRD.

FDA APPROVAL TIMELINE

January 12, 2018

On October 12, 2017, the FDA's advisory committee voted unanimously to recommend approval of voretigene neparvovec to treat vision loss due to confirmed biallelic *RPE65*-mediated IRD.

✓ Breakthrough therapy ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 0	$ 39	$ 54	$ 57	$ 53

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. The FDA has issued final and draft guidance, but regulatory hurdles remain. In February 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.

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 are 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.

PLACE IN THERAPY

The patents of several biologic drugs are set to expire in the next few years, opening up the US market for biosimilar entry; however, patent litigation and a 180-day clock after FDA approval can result in significant delays before an FDA-approved biosimilar can launch. 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.

A total of 7 biosimilars have received FDA approval. Of these, only 3 have entered the market: Sandoz' Zarxio® filgrastim-sndz, Pfizer/Celltrion's Inflectra™ infliximab-dyyb, and Merck's Renflexis™ infliximab-abda. Eli Lilly's Basaglar® insulin glargine, a follow-on agent to Sanofi's Lantus®, is also available. Amgen's Amjevita™ adalimumab-atto and Mvasi™ bevacizumab-awwb, Boehringer Ingelheim's Cyltezo™ adalimumab-adbm, and Sandoz' Erelzi™ etanercept-szzs are approved but not commercially available yet.

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.

The global biologic market is projected to exceed $390 billion by 2020. 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 analysis by the Moran Company projects biosimilars can save the government an estimated $11.4 billion by 2027, but it would require the Centers for Medicare and Medicaid Services (CMS) to revise its current reimbursement policy for biosimilars.

Biosimilar products may provide an opportunity to increase access to important biologic therapies that may increase survival and/or quality of life for many patients with diseases difficult to treat, while also reducing costs.

Blood modifier
filgrastim *SC*

Adello and Apotex are seeking approval of 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 AML; with nonmyeloid malignancies undergoing myeloablative chemotherapy followed by bone marrow transplantation; to mobilize autologous hematopoietic progenitor cells for collection by leukapheresis; in symptomatic patients with 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
Quarter 2, 2018

Apotex (Grastofil)
Pending

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 360	$290	$ 244	$ 207	$ 182

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

Immunology
infliximab (PF-06438179) *IV*

Pfizer

PF-06438179 is a tumor necrosis factor-alpha (TNF-α) inhibitor biosimilar to Janssen's Remicade®, indicated to treat RA, ankylosing spondylitis, Crohn's disease, plaque psoriasis, psoriatic arthritis, and ulcerative colitis. Clinical trials reported equivalent efficacy and similar safety of PF-06438179 to Remicade in patients with moderate to severe RA.

FDA APPROVAL TIMELINE

December, 2017 to January, 2018

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 4,452	$ 3,761	$ 2,965	$ 2,323	$ 1,808

The forecast is a projection of total US sales per year for the *branded 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 in patients acutely exposed to myelosuppressive doses of radiation (HSARS).

FDA APPROVAL TIMELINE

Pending

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 3,899	$ 3,769	$ 3,363	$ 3,028	$ 2,795

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

Oncology

rituximab *IV*

GP2013 and Truxima are biosimilars to Genentech's Rituxan®, a CD20-directed cytolytic antibody indicated for the treatment of non-Hodgkin's lymphoma (NHL), chronic lymphocytic leukemia (CLL), RA, granulomatosis with polyangiitis (GPA), and microscopic polyangiitis (MPA).

FDA APPROVAL TIMELINE

Celltrion/ Teva (Truxima)
Quarter 1, 2018

Novartis/ Sandoz (GP2013)
Quarter 2, 2018

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 3,997	$ 3,293	$ 2,640	$ 2,037	$ 1,692

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

Oncology

trastuzumab *injectable*

ABP 980, Herzuma, and HERMyl 1401O are biosimilars 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

Amgen (ABP 980)
May 31, 2018

Biocon/ Mylan (HERMyl 1401O)
December 3, 2017

- On July 13, 2017, the FDA Oncologic Drugs Advisory Committee voted 16-0 in support of approval of HERMyl 1401O for the *eligible* indication of originator product, Herceptin. This includes HER2-positive breast cancer in metastatic and adjuvant settings.

Teva (Herzuma)
March to April, 2018

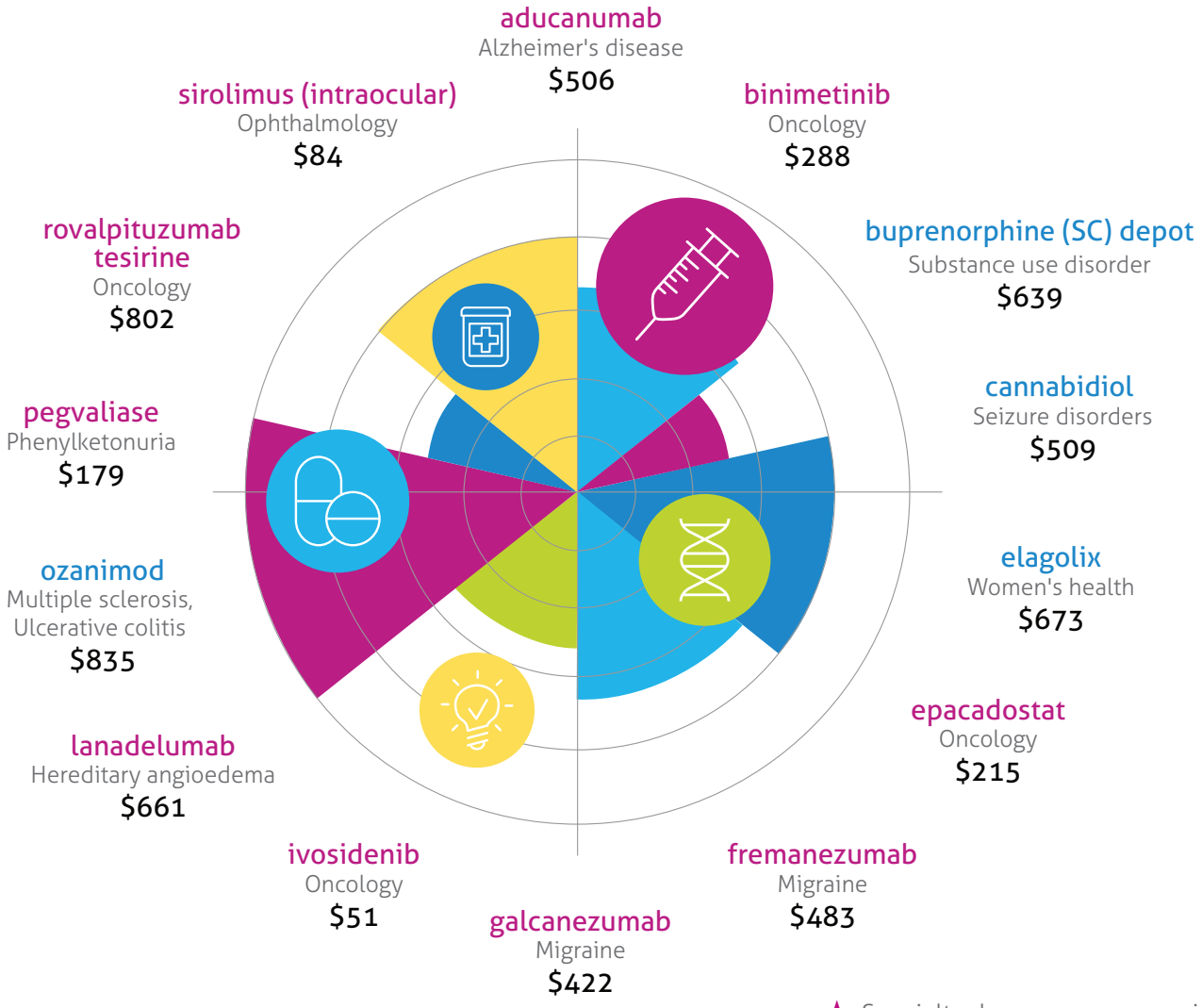
FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 2,615	$ 2,472	$ 2,270	$ 1,793	$ 1,451

The forecast is a projection of total US sales per year for the *branded 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 2021 are displayed. The financials are projected total annual US sales, reported in *millions.*



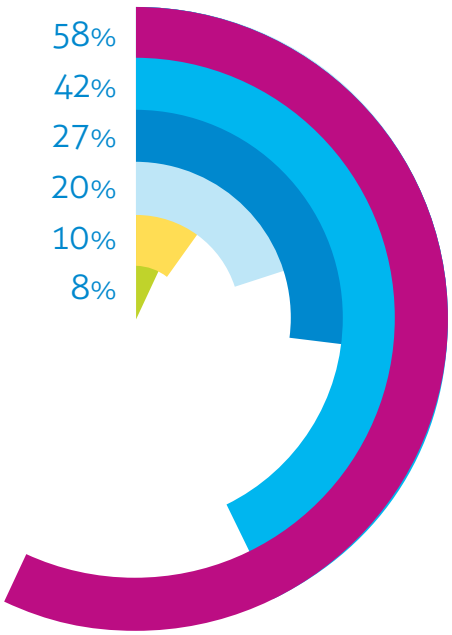
★ Specialty drug names appear in magenta throughout the publication.

Magellan Rx MANAGEMENT℠

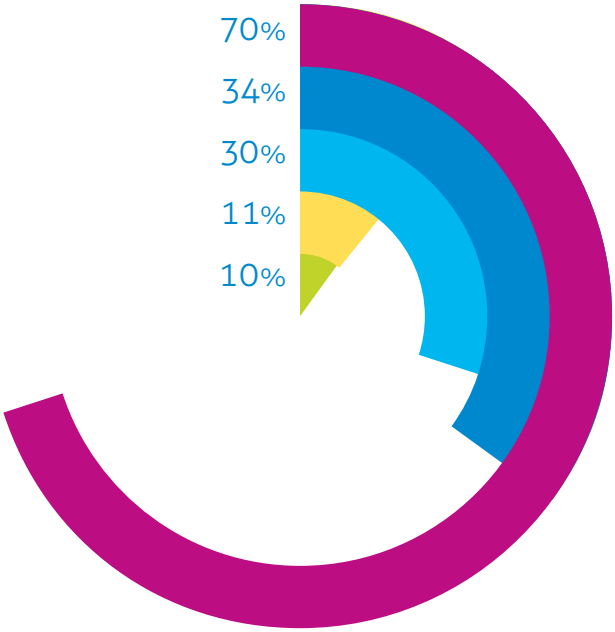
Pipeline Drug List

An aerial outline of drugs with anticipated FDA approval through 2018. 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 | Orphan drug | Priority review | 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	EXPECTED FDA APPROVAL
benralizumab	AstraZeneca	Asthma (severe, uncontrolled)	SC	Submitted - BLA	Q4, 2017
cinacalcet (Sensipar®)	Amgen	Hyperparathyroidism (pediatrics)	Oral	Submitted - sNDA; Orphan drug	Q4, 2017
fibrin sealant	Grifols	Hemostasis	Topical	Submitted - BLA	Q4, 2017
ivabradine (Corlanor®)	Amgen	Congestive heart failure/ cardiomyopathies (pediatrics)	Oral	Submitted - sNDA	Q4, 2017
rizatriptan	Redhill	Migraine	SL	Submitted - NDA	H2, 2017
eptacog beta	LFB Group	Hemophilia A and B (with inhibitors)	IV	Submitted - BLA	Oct-Nov, 2017
golimumab (Simponi Aria®)	Janssen	AS; PsA	IV	Submitted - sBLA	10/20/2017
testosterone auto-injector	Antares	Hypogonadism	SC	Submitted - NDA	10/20/2017
eculizumab (Soliris®)	Alexion	Myasthenia gravis	IV	Submitted - sBLA; Orphan drug	10/23/2017
ataluren	PTC	Duchenne muscular dystrophy	Oral	Submitted - NDA; Fast track; Orphan drug	10/24/2017
herpes zoster vaccine	GlaxoSmithKline	Shingles prevention	IM	Submitted - BLA	10/24/2017
rolapitant (Varubi®)	Tesaro	Chemotherapy induced nausea and vomiting	IV	Submitted - sNDA	10/25/2017
rivaroxaban 10 mg (Xarelto®)	Janssen	Thromboembolism risk reduction (post 6 months of standard anticoagulation)	Oral	Submitted - sNDA	10/27/2017
pemetrexed disodium, ready-to-dilute (RTD)	Eagle	NSCLC; Mesothelioma	IV	Submitted - NDA	10/30/2017
exenatide auto-injector (Bydureon®)	AstraZeneca	T2DM	SC	Submitted - sNDA	Nov-Dec, 2017
lacosamide (Vimpat®)	UCB	Partial onset seizures (≥ 4 years of age)	IV, Oral	Submitted - sNDA	Nov-Dec, 2017
ferric citrate (Auryxia®)	Keryx	Anemia due to CKD (dialysis-independent)	Oral	Submitted - sNDA	11/06/2017
letermovir	Merck	Cytomegalovirus infection	Oral	Submitted - NDA; Fast track; Orphan drug; Priority review	11/08/2017
dasatinib (Sprycel®)	Bristol-Myers Squibb	Chronic myeloid leukemia (Ph+, pediatrics)	Oral	Submitted - sNDA; Orphan drug; Priority review	11/09/2017
aprepitant	Heron	Chemotherapy induced nausea and vomiting	IV	Submitted - NDA	11/10/2017
hepatitis B vaccine	Dynavax/ Pfizer	Hepatitis B prevention	IM	Submitted - BLA	11/10/2017

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
beta-glucuronidase (human, recombinant)	Ultragenyx	Mucopolysaccharidosis VII (Sly syndrome)	IV	Submitted - BLA; Fast track; Orphan drug; Priority review	11/16/2017
aripiprazole ingestible sensor (Abilify Proteus)	Otsuka	Bipolar disorder; MDD; Schizophrenia	Oral	Submitted - NDA	11/23/2017
axicabtagene ciloleucel	Gilead/ Kite	NHL	IV	Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review	11/29/2017
alectinib (Alecensa®)	Roche	NSCLC (advanced or metastatic ALK+, 1st-line)	Oral	Submitted - sNDA; Breakthrough therapy; Orphan drug; Priority review	11/30/2017
buprenorphine hydrochloride depot	Indivior	Substance use disorder	SC	Submitted - NDA; Fast track; Priority review	11/30/2017
dengue vaccine	Sanofi	Dengue fever prevention	SC	Submitted - BLA; Fast track	December, 2017
ertugliflozin	Merck/ Pfizer	T2DM	Oral	Submitted - NDA	December, 2017
ertugliflozin/ sitagliptin	Merck/ Pfizer	T2DM	Oral	Submitted - NDA	December, 2017
solifenacin succinate (Vesicare®)	Astellas	Overactive bladder (pediatrics)	Oral	Submitted - sNDA	December, 2017
tofacitinib citrate (Xeljanz®/ Xeljanz XR)	Pfizer	PsA	Oral	Submitted - sNDA	December, 2017
infliximab (biosimilar to Janssen's Remicade)	Pfizer	RA; AS; PSO; PsA; CD; UC	IV	Submitted - BLA	Dec, 2017 - Jan, 2018
evolocumab (Repatha®)	Amgen	Major cardiovascular event risk reductions	SC	Submitted - sBLA; Priority review	12/02/2017
trastuzumab (biosimilar to Genentech's Herceptin)	Biocon/ Mylan	Breast cancer (HER2+); Gastric and gastroesophageal cancer (HER2+)	IV	Submitted - BLA	12/03/2017
semaglutide	Novo Nordisk	T2DM	SC	Submitted - NDA	12/05/2017
vemurafenib (Zelboraf®)	Genentech	Erdheim-Chester disease (BRAF V600 mutation)	Oral	Submitted - sNDA; Breakthrough therapy; Orphan drug; Priority review	12/07/2017
brentuximab vedotin (Adcetris®)	Seattle Genetics	Cutaneous T cell lymphoma	IV	Submitted - sBLA; Breakthrough therapy; Orphan drug; Priority review	12/15/2017
hydrogen peroxide	Aclaris	Seborrheic keratosis	Topical	Submitted - NDA	12/22/2017

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
obinutuzumab (Gazyva®)	Genentech	Follicular lymphoma (1st-line)	IV	Submitted - sBLA	12/22/2017
sirolimus	Santen	Uveitis	Intraocular	Submitted - NDA; Orphan drug	12/22/2017
testosterone undecanoate	Clarus	Hypogonadism	Oral	Submitted - NDA	12/26/2017
macimorelin acetate	Aeterna Zentaris	Growth hormone deficiency evaluation (adults)	Oral	Submitted - NDA; Orphan drug	12/29/2017
sunitinib malate (Sutent®)	Pfizer	RCC (adjuvant)	Oral	Submitted - sNDA	January, 2018
acalabrutinib	AstraZeneca	Mantle cell lymphoma	Oral	Submitted - NDA; Breakthrough therapy; Orphan drug; Priority review	Jan-Feb, 2018
polyethylene glycol (low-volume)	Valeant	Bowel cleansing	Oral	Submitted - NDA	Q1, 2018
rituximab (biosimilar to Genentech's Rituxan)	Celltrion/ Teva	CCL/SLL; Indolent NHL; Antineutrophil Cytoplasmic Antibodies (ANCA) Associated Vasculitis; RA	IV	Submitted - BLA	Q1, 2018
ibalizumab	Theratechnologies	HIV-1 infection (multi-drug resistant)	IV	Submitted - BLA; Breakthrough therapy; Fast track; Orphan drug; Priority review	01/03/2018
voretigene neparvovec	Spark	Inherited retinal disorder (biallelic *RPE65*-mediated)	Intraocular	Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review	01/12/2018
quadrivalent influenza vaccine (Fluarix® Quadrivalent)	GlaxoSmithKline	Influenza prevention (ages 6-35 months)	IM	Submitted - sBLA	01/15/2018
buprenorphine depot	Apple Tree	Substance use disorder	SC	Submitted - NDA; Fast track; Priority review	01/19/2018
plecanatide (Trulance®)	Synergy	IBS with constipation	Oral	Submitted - sNDA	01/24/2018
ciprofloxacin (liposomal, dual-release)	Grifols	Bronchiectasis	Inhaled	Submitted - NDA; Fast track; Orphan drug; Priority review	01/26/2018
epinephrine 0.1 mg auto-injector (Auvi-Q®)	Kaleo	Anaphylaxis (infants/ small children)	IM, SC	Submitted - sNDA; Priority review	01/26/2018
lutetium Lu 177 dotatate	Advanced Accelerator Applications	Neuroendocrine tumors	IV	Submitted - NDA; Fast track; Orphan drug	01/27/2018

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
lurasidone (Latuda®)	Sumitomo Dainippon	Bipolar disorder (ages 10-17 years)	Oral	Submitted - sNDA	Feb-Mar, 2018
tofacitinib citrate (Xeljanz/ Xeljanz XR)	Pfizer	UC	Oral	Submitted - sNDA	Feb-Mar, 2018
rilpivirine/ dolutegravir	GlaxoSmithKline	HIV-1 infection	Oral	Submitted - NDA	02/01/2018
andexanet alfa	Portola	Anticoagulant reversal	IV	Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review	02/02/2018
denosumab (Xgeva®)	Amgen	Fracture prevention associated with bone metastases from multiple myeloma	SC	Submitted - sBLA	02/02/2018
testosterone undecanoate	Lipocine	Hypogonadism	Oral	Submitted - NDA	02/09/2018
bictegravir/ emtricitabine/ tenofovir alafenamide	Gilead	HIV-1 infection	Oral	Submitted - NDA; Orphan drug; Priority review	02/12/2018
dexamethasone sustained-release	Icon	Ocular pain and inflammation associated with cataract surgery	Intraocular	Submitted - NDA	02/12/2018
hydroxyprogesterone caproate (Makena® auto-injector)	AMAG	Preterm birth prevention	SC	Submitted - sNDA; Orphan drug	02/14/2018
alprostadil	Apricus	Erectile dysfunction	Topical	Submitted - NDA	02/17/2018
emicizumab	Roche	Hemophilia A (factor VIII inhibition)	SC	Submitted - BLA; Breakthrough therapy; Orphan drug; Priority review	02/23/2018
lidocaine patch	Sorrento	Postherpetic neuralgia	Transdermal	Submitted - NDA	02/28/2018
LJPC-501	La Jolla	Hypotension (vasodilatory shock)	N/A	Submitted - NDA; Priority review	02/28/2018
netarsudil	Aerie	Glaucoma; Ocular hypertension	Ophthalmic	Submitted - NDA	02/28/2018
tezacaftor/ ivacaftor	Vertex	CF	Oral	Submitted - NDA; Breakthrough therapy; Orphan drug; Priority review	02/28/2018
tildrakizumab	Sun	PSO	SC	Submitted - BLA	Mar-Apr, 2018
trastuzumab (biosimilar to Genentech's Herceptin)	Teva	Breast cancer (HER2+); Gastric and gastroesophageal cancer (HER2+)	IV	Submitted - BLA	Mar-Apr, 2018
immune globulin (human) (Hizentra®)	CSL	Chronic inflammatory demyelinating polyneuropathy	SC	Submitted - sBLA; Orphan drug	Mar-May, 2018

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
ciprofloxacin (Otiprio®)	Ostonomy	Acute otitis media	Intratympanic	Submitted - sNDA	03/02/2018
HIV vaccine	Immune Response	HIV-1 infection	IM	Submitted - BLA; Orphan drug	03/30/2018
filgrastim (biosimilar to Amgen's Neupogen)	Adello	Neutropenia/ leukopenia	SC	Submitted - BLA	Q2, 2018
rituximab (biosimilar to Genentech's Rituxan)	Novartis/ Sandoz	CLL/SLL; Indolent NHL; Antineutrophil cytoplasmic antibodies associated vasculitis; RA	IV	Submitted - BLA	Q2, 2018
ulipristal acetate	Allergan	Uterine fibroids	Oral	Submitted - NDA	H2, 2018
netupitant/ palonosetron (Akynzeo®)	Helsinn	Chemotherapy induced nausea and vomiting	IV	Submitted - sNDA	Apr-May, 2018
ixekizumab (Taltz®)	Eli Lilly	PsA	SC	Submitted - sBLA	04/13/2018
fostamatinib disodium	Rigel	Immune thrombocytopenic purpura	Oral	Submitted - NDA; Orphan drug	04/17/2018
burosumab	Ultragenyx	X-linked hypophosphatemia	SC	Submitted - BLA; Breakthrough therapy; Fast track; Orphan drug	04/24/2018
mepolizumab (Nucala®)	GlaxoSmithKline	Antineutrophil cytoplasmic antibodies associated vasculitis	IV, SC	Submitted - sBLA	04/27/2018
solifenacin/ mirabegron	Astellas	Overactive bladder	Oral	Submitted - NDA	04/30/2018
erenumab	Amgen/ Novartis	Migraine	SC	Submitted - BLA	05/17/2018
fluticasone furoate (Arnuity® Ellipta®)	GlaxoSmithKline	Asthma (ages 5-11 years)	Inhaled	Submitted - sNDA	05/24/2018
certolizumab (Cimzia®)	UCB	PSO	SC	Submitted - sBLA	05/25/2018
lenvatinib (Lenvima®)	Eisai	HCC (1st-line)	Oral	Submitted - sNDA; Orphan drug	05/25/2018
denosumab (Prolia®)	Amgen	Glucocorticoid-induced osteoporosis	SC	Submitted - sBLA	05/28/2018
celecoxib/ amlodipine besylate	Kitov	Hypertension; Osteoarthritis	Oral	Submitted - NDA	05/31/2018
meloxicam	Recro	Postsurgical pain	IV	Submitted - NDA	05/31/2018
trastuzumab (biosimilar to Genentech's Herceptin)	Amgen	Breast cancer (HER2+); Gastric and gastroesophageal cancer (HER2+)	IV	Submitted - BLA	05/31/2018
ferumoxytol (Feraheme®)	AMAG	Anemia (iron deficiency)	IV	Submitted - sNDA; Priority review	06/03/2018
cabozantinib (Cabometyx™/ Cometriq®)	Exelixis	RCC (1st-line)	Oral	Submitted - sNDA	06/15/2018
pegvaliase	Biomarin	Phenylketonuria (PKU)	SC	Submitted - BLA; Orphan drug; Priority review	06/29/2018

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
binimetinib	Array	Melanoma	Oral	Submitted - NDA	07/05/2018
encorafenib	Array	Melanoma	Oral	Submitted - NDA	07/05/2018
halobetasol propionate/ tazarotene	Valeant	PSO	Topical	Submitted - NDA	07/05/2018
buprenorphine spray	Insys	Acute pain (moderate to severe)	SL	Submitted - NDA	07/27/2018
canagliflozin (Invokana®)	Janssen	Cardiovascular risk reduction in patients with T2DM	Oral	Submitted - sNDA	08/02/2018
canagliflozin/ metformin (Invokamet®, Invokamet XR)	Janssen	Cardiovascular risk reduction in patients with T2DM	Oral	Submitted - sNDA	08/02/2018
rucaparib (Rubraca®)	Clovis	Ovarian/fallopian tube/ peritoneal cancer (maintenance)	Oral	Submitted - sNDA	08/09/2018
damoctocog alfa pegol	Bayer	Hemophilia A	IV	Submitted - BLA	08/31/2018
dasotraline	Sumitomo Dainippon	ADHD	Oral	Submitted - NDA	08/31/2018
volanesorsen	Akcea	Dyslipidemia/ hypercholesterolemia	SC	Submitted - NDA; Orphan drug	08/31/2018
elagolix	Abbvie	Endometriosis	Oral	Submitted - NDA	09/06/2018
avatrombopag	Dova	Thrombocytopenia (with chronic liver disease)	Oral	Submitted - NDA	09/22/2018
apalutamide	Janssen	Prostate cancer (castration-resistant, non-metastatic)	Oral	Submitted - NDA	10/11/2018
filgrastim (biosimilar to Amgen's Neupogen)	Apotex	Neutropenia/ leukopenia	SC	Submitted - BLA	Pending
gepirone ER	GlaxoSmithKline	MDD	Oral	Submitted - NDA	Pending
pegfilgrastim (biosimilar to Amgen's Neulasta)	Apotex	Neutropenia/ leukopenia	SC	Submited - sBLA	Pending
plasminogen (human)	Prometic Life Sciences	Hypoplasminogenemia	IV	Submitted - BLA; Fast track; Orphan drug	Pending
abemaciclib (Verzenio®)	Shire	NSCLC	Oral	Phase 3 - sNDA	TBD
acalabrutinib	AstraZeneca	CLL/SLL	Oral	Phase 3 - NDA; Orphan drug	TBD
aducanumab	Biogen	Alzheimer's disease	IV	Phase 3 - BLA; Fast track	TBD
adagloxad simolenin	OBI	Breast cancer	SC	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)	Kyowa Hakko Kirin	RA; AS; PSO; PsA: JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Merck	RA; AS; PSO; PsA: JIA; CD; UC	SC	Phase 3 - BLA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
adalimumab (biosimilar to Abbvie's Humira)	Momenta	RA; AS; PSO; PsA: JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Mylan	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
adalimumab (biosimilar to Abbvie's Humira)	Samsung Bioepis	RA; AS; PSO; PsA: JIA; CD; UC	SC	Phase 3 - BLA	TBD
adalimumab (biosimilar to Abbvie's Humira)	Sandoz	RA; AS; PSO; PsA: JIA; CD; UC	SC	Phase 3 - BLA	TBD
afamelanotide	Clinuvel	Porphyria	Intradermal	Phase 3 - NDA; Fast track; Orphan drug	TBD
alicaforsen	Atlantic Healthcare	UC	Rectal	Phase 3 - BLA; Fast track; Orphan drug	TBD
allopregnanolone	Sage	MDD	IV	Phase 3 - NDA; Breakthrough therapy	TBD
alpelisib	Novartis	Breast cancer	Oral	Phase 3 - NDA	TBD
amantadine ER	Osmotica	Levodopa-induced dyskinesia	Oral	Phase 3 - NDA; Orphan drug	TBD
amifampridine	Catalyst	Lambert-Eaton myasthenic syndrome; Myasthenia gravis; Congenital myasthenic syndrome	Oral	Phase 3 - NDA; Breakthrough therapy; Orphan drug	TBD
amrubicin	Celgene	SCLC	IV	Phase 3 - NDA; Fast track; Orphan drug	TBD
andecaliximab	Gilead	Gastric cancer	IV	Phase 3 - BLA; Orphan drug	TBD
andolast	Mylan	Asthma	Inhaled	Phase 3 - NDA	TBD
anifrolumab	AstraZeneca	SLE	IV, SC	Phase 3 - BLA; Fast track	TBD
anlotinib hydrochloride	Advenchen	Sarcoma	Oral	Phase 3 - NDA; Orphan drug	TBD
apatinib mesylate	LSK Biopartners	Gastric cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
apremilast (Otezla®)	Celgene	Behçet syndrome	Oral	Phase 3 - sNDA; Orphan drug	TBD
astodrimer sodium	Starpharma	Urinary/reproductive tract infections	Intravaginal	Phase 3 - NDA; Fast track	TBD
atezolizumab (Tecentriq®)	Genentech	RCC; Breast cancer; Ovarian cancer; CRC; Prostate cancer; SCLC; Melanoma	IV	Phase 3 - sBLA; Orphan drug	TBD
avacopan	Chemocentryx	Antineutrophil cytoplasmic antibodies-associated vasculitis	Oral	Phase 3 - NDA; Orphan drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
avelumab (Bavencio®)	Merck	NSCLC; Ovarian cancer; Gastric cancer; RCC; DLBCL; SCCHN	IV	Phase 3 - sBLA	TBD
axalimogene filolisbac	Advaxis	Cervical cancer	IV	Phase 3 - BLA; Fast track; Orphan drug	TBD
azeliragon	vTv	Alzheimer's disease	Oral	Phase 3 - NDA; Fast track	TBD
baclofen/ naltrexone/ sorbitol	Pharnext	Charcot-Marie-Tooth disease	Oral	Phase 3 - NDA	TBD
baricitinib	Eli Lilly	RA	Oral	Phase 3 - NDA	TBD
bempedoic acid	Esperion	Dyslipidemia/ hypercholesterolemia	Oral	Phase 3 - NDA	TBD
bendamustine (Treanda®)	Teva	Mantle cell lymphoma	IV	Phase 3 - sNDA	TBD
benralizumab	AstraZeneca	COPD	SC	Phase 3 - BLA	TBD
beperminogene perplasmid	Mitsubishi Tanabe	Peripheral T cell lymphoma	IM	Phase 3 - BLA; Fast track	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Biocon/ Mylan	CRC; NSCLC; Ovarian/fallopian tube/ peritoneal cancer; Glioblastoma; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Boehringer Ingelheim	CRC; NSCLC; Ovarian/fallopian tube/ peritoneal cancer; Glioblastoma; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	International Biotechnology Center Generium	CRC; NSCLC; Ovarian/fallopian tube/ peritoneal cancer; Glioblastoma; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Kyowa Hakko Kirin	CRC; NSCLC; Ovarian/fallopian tube/ peritoneal cancer; Glioblastoma; RCC	IV	Phase 3 - BLA	TBD
bevacizumab (biosimilar to Genentech's Avastin)	Pfizer	CRC; NSCLC; Ovarian/fallopian tube/ peritoneal cancer; Glioblastoma; RCC	IV	Phase 3 - BLA	TBD
bexagliflozin	Theracos	T2DM	Oral	Phase 3 - NDA	TBD
binimetinib	Array	CRC	Oral	Phase 3 - NDA	TBD
blinatumomab (Blincyto®)	Amgen	DLBCL	IV	Phase 3 - sBLA	TBD
bortezomib (Velcade®)	Takeda	DLBCL	IV, SC	Phase 3 - sNDA	TBD
bremelanotide	AMAG	Female sexual arousal disorder	SC	Phase 3 - NDA	TBD
brentuximab vedotin (Adcetris®)	Seattle Genetics	Peripheral T cell lymphoma	IV	Phase 3 - sBLA; Fast track; Orphan drug	TBD
brexpiprazole (Rexulti®)	Otsuka	Alzheimer's disease	Oral	Phase 3 - sNDA; Fast track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
brincidofovir	Chimerix	Adenovirus infection; Cytomegalovirus infection	Oral	Phase 3 - NDA; Fast track	TBD
brolucizumab	Novartis	Wet AMD	Intravitreal	Phase 3 - BLA	TBD
cabozantinib (Cabometyx®/ Cometriq®)	Exelixis	HCC	Oral	Phase 3 - sNDA; Orphan drug	TBD
calaspargase pegol	Shire	AML	IV	Phase 3 - BLA	TBD
canakinumab (Ilaris®)	Novartis	Atherosclerosis	IV, SC	Phase 3 - sBLA	TBD
cannabidiol	GW	Dravet syndrome; Lennox-Gastaut syndrome; Tuberous sclerosis complex; Infantile spasms (West syndrome)	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
cannabidiol oral solution	Insys	Dravet syndrome; Lennox-Gastaut syndrome	Oral	Phase 3 - NDA	TBD
caplacizumab	Ablynx	Thrombotic thrombocytopenic purpura	IV, SC	Phase 3 - BLA; Orphan drug	TBD
carotuximab	Tracon	Sarcoma	IV	Phase 3 - BLA; Orphan drug	TBD
cediranib	AstraZeneca	Ovarian cancer; Biliary tract cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
cefiderocol	Shionogi	Urinary/reproductive tract; Septicemia/ bacteremia; Hospital acquired pneumonia	IV	Phase 3 - NDA	TBD
ceftazidime/ avibactam (Avycaz®)	Allergan	Hospital acquired pneumonia	IV	Phase 3 - sNDA; Fast track	TBD
cemiplimab	Regeneron	NSCLC	IV	Phase 3 - BLA	TBD
ciprofloxacin dry powder inhaler	Bayer	Bronchiectasis	Inhaled	Phase 3 - NDA; Orphan drug	TBD
cobimetinib (Cotellic®)	Genentech	CRC	Oral	Phase 3 - sNDA	TBD
crenolanib besylate	AROG	Gastrointestinal stromal tumor (GIST)	Oral	Phase 3 - NDA; Fast track	TBD
cytomegalovirus vaccine	Astellas	Cytomegalovirus prevention	IM	Phase 3 - BLA; Orphan drug	TBD
dapagliflozin (Farxiga®)	AstraZeneca	T1DM; Renal and cardiovascular outcomes in patients with CKD	Oral	Phase 3 - sNDA	TBD
daprodustat	GlaxoSmithKline	Anemia related to CKD	Oral	Phase 3 - NDA	TBD
darleukin	Philogen	Melanoma	IV	Phase 3 - BLA	TBD
darolutamide	Bayer	Prostate cancer	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
darunavir/ emtricitabine/ tenofovir alafenamide/ cobicistat	Janssen	HIV-1 infection	Oral	Phase 3 - NDA	TBD
dasiprotimut-T	Accentia	Indolent NHL	SC	Phase 3 - BLA; Fast track; Orphan drug	TBD
denileukin diftitox	Dr. Reddy's	Peripheral T cell lymphoma	IV	Phase 3 - BLA	TBD
derazantinib	Arqule	Biliary tract cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
difelikefalin	Cara	Uremic pruritus; Osteoarthritis pain	IV	Phase 3 - NDA; Breakthrough therapy	TBD
dinutuximab (Unituxin®)	United Therapeutics	SCLC	IV	Phase 3 - sBLA	TBD
dolutegravir/ lamivudine	GlaxoSmithKline	HIV-1 infection	Oral	Phase 3 - NDA	TBD
donor lymphocytes depleted alloreactive T cells	Kiadis	AML	IV	Phase 3 - BLA	TBD
doravirine	Merck	HIV-1 infection	Oral	Phase 3 - NDA	TBD
dovitinib lactate	Novartis	Breast cancer	Oral	Phase 3 - NDA	TBD
dupilumab (Dupixent®)	Regeneron	Asthma (severe, uncontolled); Nasal polyposis	SC	Phase 3 - sBLA	TBD
durvalumab (Imfinzi™)	AstraZeneca	NSCLC; SCCHN; SCLC	IV	Phase 3 - sBLA; Breakthrough therapy; Fast track	TBD
dusquetide	Soligenix	Mucositis	IV	Phase 3 - NDA; Fast track	TBD
duvelisib	Verastem	CLL/SLL; Indolent NHL	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
eculizumab (Soliris®)	Alexion	Neuromyelitis optica (Devic's syndrome); Delayed graft function	IV	Phase 3 - sBLA; Orphan drug	TBD
eflapegrastim	Spectrum	Neutropenia/ leukopenia	SC	Phase 3 - NDA	TBD
elafibranor	Genfit	Non-alcoholic steatohepatitis	Oral	Phase 3 - NDA; Fast track	TBD
elagolix	Abbvie	Uterine fibroids	Oral	Phase 3 - NDA	TBD
encorafenib	Array	CRC	Oral	Phase 3 - NDA	TBD
ensartinib	Xcovery	NSCLC	Oral	Phase 3 - NDA	TBD
entinostat	Syndax	Breast cancer	Oral	Phase 3 - NDA; Breakthrough therapy	TBD
epacadostat	Incyte	Melanoma	Oral	Phase 3 - NDA	TBD
epoetin alfa (biosimilar to Janssen's Procrit®)	Novartis	Anemia related to CKD	IV, SC	Phase 3 - BLA	TBD
epratuzumab	Immunomedics	AML	IV	Phase 3 - BLA; Orphan drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
eptinezumab	Alder	Migraine	IV	Phase 3 - BLA	TBD
eravacycline	Tetraphase	Intra-abdominal infections; Urinary/ reproductive tract infections	IV, Oral	Phase 3 - NDA; Fast track	TBD
erdosteine	Alitair	COPD	Oral	Phase 3 - NDA	TBD
esketamine	Janssen	MDD	Intranasal	Phase 3 - NDA; Breakthrough therapy; Fast track	TBD
etanercept (biosimilar to Amgen's Enbrel®)	Coherus	RA; JIA; AS; PSO; PsA	SC	Phase 3 - BLA	TBD
etanercept (biosimilar to Amgen's Enbrel)	Samsung Bioepis	RA; JIA; AS; PSO; PsA	SC	Phase 3 - BLA	TBD
etrolizumab	Genentech	CD; UC	IV, SC	Phase 3 - BLA; Orphan drug	TBD
fenfluramine	Zogenix	Dravet syndrome	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
fevipiprant	Novartis	Asthma	Oral	Phase 3 - NDA	TBD
filgotinib	Gilead	RA; CD; UC	Oral	Phase 3 - NDA	TBD
fingolimod (Gilenya®)	Novartis	MS (pediatrics)	Oral	Phase 3 - sNDA	TBD
fluticasone furoate/ umeclidinium bromide/ vilanterol (Trelegy® Ellipta®)	GlaxoSmithKline	Asthma	Inhaled	Phase 3 - sNDA	TBD
fosbretabulin tromethamine	Mateon	Ovarian cancer	IV	Phase 3 - NDA; Fast track; Orphan drug	TBD
fosmetpantotenate	Retrophin	Pantothenate kinase-associated neurodegeneration (PKAN)	IV	Phase 3 - NDA; Fast track; Orphan drug	TBD
fremanezumab	Teva	Migraine; Cluster headache	IV, SC	Phase 3 - BLA	TBD
furosemide pump	scPharmaceuticals	Heart failure	SC	Phase 3 - NDA	TBD
fusidic acid	Cempra	Skin and skin-structure infections; Bone and joint infections	Oral	Phase 3 - NDA; Orphan drug	TBD
galcanezumab	Eli Lilly	Migraine; Cluster headache	SC	Phase 3 - BLA; Fast track	TBD
galunisertib	Eli Lilly	Myelodysplastic syndrome	Oral	Phase 3 - NDA	TBD
gilteritinib	Astellas	AML	Oral	Phase 3 - NDA; Orphan drug	TBD
glycopyrronium bromide (Seebri™ Neohaler®)	Sumitomo Dainippon	Asthma	Inhaled	Phase 3 - sNDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
grazoprevir/ elbasvir (Zepatier®)	Merck	HCV infection in patients with CKD	Oral	Phase 3 - sNDA; Breakthrough therapy	TBD
guadecitabine	Otsuka	Myelodysplastic syndrome; AML	SC	Phase 3 - NDA; Orphan drug	TBD
human growth hormone (CTP-modified, long-acting)	Opko	Growth hormone deficiency	SC	Phase 3 - BLA; Orphan drug	TBD
hypericin	Soligenix	Cutaneous T cell lymphoma	Topical	Phase 3 - NDA; Fast track; Orphan drug	TBD
ibritumomab tiuxetan	Spectrum	DLBCL	IV	Phase 3 - BLA	TBD
ibrutinib (Imbruvica®)	Abbvie	Indolent NHL; DLBCL; Pancreatic cancer; Myelodysplastic syndrome	Oral	Phase 3 - sNDA; Orphan drug	TBD
iclaprim	Motif Bio	Skin and skin-structure infections; Hospital acquired pneumonia	IV	Phase 3 - NDA; Fast track	TBD
icosapent ethyl (Vascepa®)	Amarin	Major cardiovascular event risk reductions	Oral	Phase 3 - sNDA	TBD
idasanutlin	Roche	AML	Oral	Phase 3 - NDA	TBD
immunoglobulin 10%	Octapharma	Thrombocytopenia	IV	Phase 3 - BLA	TBD
indacaterol/ glycopyrronium bromide/ mometasone furoate	Novartis	Asthma	Inhaled	Phase 3 - NDA	TBD
inebilizumab	AstraZeneca	Neuromyelitis optica (Devic's syndrome)	IV	Phase 3 - BLA; Orphan drug	TBD
infliximab (biosimilar to Janssen's Remicade)	Amgen	RA; AS; PSO; PsA; CD; UC	IV	Phase 3 - BLA	TBD
inotersen	GlaxoSmithKline	Familial amyloid polyneuropathy	SC	Phase 3 - NDA; Fast track; Orphan drug	TBD
insulin glargine (follow-on to Sanofi's Lantus)	Biocon/ Mylan	T1DM; T2DM	SC	Phase 3 - NDA	TBD
insulin lispro (follow-on to Eli Lilly's Humalog®)	Sanofi	T1DM; T2DM	SC	Phase 3 - NDA	TBD
ipatasertib	Genentech	Prostate cancer	Oral	Phase 3 - NDA	TBD
isatuximab	Sanofi	Multiple myeloma	IV	Phase 3 - BLA; Orphan drug	TBD
ivosidenib	Agios	AML; Biliary tract cancer	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
lampalizumab	Roche	Dry AMD	Intravitreal	Phase 3 - BLA; Fast track	TBD
lanadelumab	Shire	Hereditary angioedema	SC	Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug	TBD
lasmiditan	Eli Lilly	Migraine	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
lefamulin	Nabriva	Community acquired pneumonia	IV, Oral	Phase 3 - NDA; Fast track	TBD
lefitolimod	Mologen	CRC	SC	Phase 3 - NDA	TBD
lenalidomide (Revlimid®)	Celgene	DLBCL; Indolent NHL; Marginal zone lymphoma	Oral	Phase 3 - sNDA; Orphan drug	TBD
lentiviral beta-globin gene transfer	Bluebird	Beta thalassemia	IV	Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug	TBD
lorlatinib	Pfizer	NSCLC	Oral	Phase 3 - NDA; Breakthrough therapy	TBD
lubiprostone (Amitiza®)	Sucampo	Chronic constipation (pediatrics)	Oral	Phase 3 - sNDA	TBD
lurbinectidin	Pharmamar	Ovarian cancer; SCLC	IV	Phase 3 - NDA; Orphan drug	TBD
luspatercept	Acceleron	Beta thalassemia; Myelodysplastic syndrome	SC	Phase 3 - BLA; Fast track; Orphan drug	TBD
lusutrombopag	Shionogi	Thrombocytopenia	Oral	Phase 3 - NDA; Fast track	TBD
margetuximab	AstraZeneca	Breast cancer	IV	Phase 3 - BLA	TBD
masitinib mesylate	AB Science	Gastrointestinal stromal tumor (GIST); Pancreatic cancer; Multiple myeloma; CRC; Prostate cancer; Melanoma; Ovarian cancer; Mastocytosis; MS (secondary progressive); Alzheimer's disease; Asthma; ALS	Oral	Phase 3 - NDA; Orphan drug	TBD
mepolizumab (Nucala®)	GlaxoSmithKline	COPD; Hypereosinophilic syndrome; Nasal polyposis	IV, SC	Phase 3 - sBLA; Orphan drug	TBD
meropenem/ vaborbactam (Vabomere®)	The Medicines Company	Hospital acquired pneumonia; Septicemia/ bacteremia	IV	Phase 3 - sNDA	TBD
microbiota suspension	Rebiotix	Clostridium difficile-associated diarrhea/ infection prevention or recurrence	Rectal	Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug	TBD
midazolam spray	Upsher-Smith	Seizure rescue	Intranasal	Phase 3 - NDA; Fast track; Orphan drug	TBD
migalastat	Amicus	Fabry's disease	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
mirvetuximab soravtansine	Immunogen	Ovarian cancer	IV	Phase 3 - BLA; Orphan drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
mongersen	Celgene	CD	Oral	Phase 3 - NDA; Orphan drug	TBD
moxetumomab pasudotox	AstraZeneca	Hairy cell leukemia	IV	Phase 3 - BLA	TBD
moxidectin	Medicines Development for Global Health	Onchocerciasis	Oral	Phase 3 - NDA	TBD
napabucasin	Sumitomo Dainippon	Pancreatic cancer; NSCLC	Oral	Phase 3 - NDA; Orphan drug	TBD
netarsudil/ latanoprost	Aerie	Glaucoma; Ocular hypertension	Ophthalmic	Phase 3 - NDA	TBD
nintedanib (Ofev®)	Boehringer Ingelheim	NSCLC; CRC; Scleroderma; Mesothelioma	Oral	Phase 3 - sNDA; Orphan drug	TBD
nitric oxide	Mallinckrodt	Bronchopulmonary dysplasia	Inhaled	Phase 3 - NDA	TBD
nivolumab (Opdivo)	Bristol-Myers Squibb	SCLC; Mesothelioma; Multiple myeloma; Glioblastoma multiforme; Anaplastic astrocytoma; Esophageal cancer; Gastric cancer	IV	Phase 3 - sBLA; Orphan drug	TBD
obeticholic acid (Ocaliva®)	Intercept	Non-alcoholic steatohepatitis	Oral	Phase 3 - sNDA; Breakthrough therapy	TBD
ofranergene obadenovec	VBL	Anaplastic astrocytoma; Glioblastoma	IV	Phase 3 - BLA; Fast track; Orphan drug	TBD
olaparib (Lynparza®)	AstraZeneca	Prostate cancer; Pancreatic cancer; Breast cancer	Oral	Phase 3 - sNDA; Breakthrough therapy	TBD
oliceridine	Trevena	Acute Pain	IV	Phase 3 - NDA; Breakthrough therapy; Fast track	TBD
olipudase alfa	Sanofi	Niemann-Pick disease	IV	Phase 3 - BLA; Breakthrough therapy; Orphan drug	TBD
olumacostat glasaretil	Dermira	Acne	Topical	Phase 3 - NDA	TBD
omadacycline	Paratek	Skin and skin-structure infections; Community acquired pneumonia	IV, Oral	Phase 3 - NDA; Fast track	TBD
osilodrostat	Novartis	Cushing's syndrome	Oral	Phase 3 - NDA; Orphan drug	TBD
ozanimod	Celgene	MS (relapsing); UC	Oral	Phase 3 - NDA	TBD
pacritinib	CTI	Myelofibrosis	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
patisiran	Alnylam	Familial amyloid polyneuropathy	IV	Phase 3 - NDA; Fast track; Orphan drug	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
peanut protein capsule	Aimmune	Peanut allergy	Oral	Phase 3 - BLA; Breakthrough therapy; Fast track	TBD
pegilodecakin	ARMO	Pancreatic cancer	SC	Phase 3 - NDA; Fast track; Orphan drug	TBD
pegunigalsidase alfa	Protalix	Fabry's disease	IV	Phase 3 - BLA	TBD
pelareorep	Oncolytics	SCCHN	IV	Phase 3 - BLA	TBD
pembrolizumab (Keytruda®)	Merck	Breast cancer; SCLC; Esophageal cancer; HCC; RCC; Multiple myeloma	IV	Phase 3 - sBLA; Breakthrough therapy	TBD
pertuzumab (Perjeta®)	Genentech	Ovarian cancer	IV	Phase 3 - sBLA	TBD
pexidartinib	Daiichi Sankyo	Pigmented villonodular synovitis	Oral	Phase 3 - NDA; Breakthrough therapy; Orphan drug	TBD
pixantrone dimaleate	CTI	DLBCL	IV	Phase 3 - NDA; Fast track; Orphan drug	TBD
plazomicin	Achaogen	Urinary/reproductive tract; Septicemia/ bacteremia; Hospital acquired pneumonia	IV	Phase 3 - NDA; Breakthrough therapy; Fast track	TBD
plinabulin	Beyondspring	NSCLC; Neutropenia/ leukopenia	IV	Phase 3 - NDA	TBD
plitidepsin	Pharmamar	Multiple myeloma	IV	Phase 3 - NDA; Orphan drug	TBD
pracinostat	Helsinn Healthcare	AML	Oral	Phase 3 - NDA; Breakthrough therapy; Orphan drug	TBD
prucalopride	Shire	Chronic idiopathic constipation	Oral	Phase 3 - NDA	TBD
quizartinib	Daiichi Sankyo	AML	Oral	Phase 3 - NDA; Orphan drug	TBD
ramucirumab (Cyramza®)	Eli Lilly	Bladder cancer; HCC	IV	Phase 3 - sBLA; Orphan drug	TBD
recombinant factor VIII (Obizur®)	Shire	Hemophilia A (factor VIII inhibitors)	IV	Phase 3 - sBLA; Fast track; Orphan drug	TBD
relugolix	Myovant Sciences	Uterine fibroids; Endometriosis; Prostate cancer	Oral	Phase 3 - NDA	TBD
remimazolam	Cosmo	Anesthesia	IV	Phase 3 - NDA	TBD
reparixin	Dompé	Transplant rejection	IV	Phase 3 - NDA; Orphan drug	TBD
revefenacin	Theravance	COPD	Inhaled	Phase 3 - NDA	TBD
rifamycin	Cosmo	Gastroenteritis	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
rigosertib	Onconova	Myelodysplastic syndrome	IV	Phase 3 - NDA; Orphan drug	TBD
risankizumab	Abbvie	PSO	IV, SC	Phase 3 - BLA	TBD
risperidone depot (monthly)	Indivior	Schizophrenia	SC	Phase 3 - NDA	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Amgen	CLL/SLL; Indolent NHL; Antineutrophil cytoplasmic antibodies associated vasculitis; RA	IV	Phase 3 - BLA	TBD
rituximab (biosimilar to Genentech's Rituxan)	Pfizer	CLL/SLL; Indolent NHL; Antineutrophil cytoplasmic antibodies associated vasculitis; RA	IV	Phase 3 - BLA	TBD
rituximab (Rituxan)	Genentech	Pemphigus vulgaris	IV	Phase 3 - sBLA; Breakthrough therapy; Orphan drug	TBD
rivipansel	Pfizer	Sickle cell anemia	IV, SC	Phase 3 - NDA; Fast track; Orphan drug	TBD
ropeginterferon alfa-2b	Pharmaessentia	Polycythemia vera	SC	Phase 3 - BLA; Orphan drug	TBD
rovalpituzumab tesirine	Abbvie	SCLC	IV	Phase 3 - BLA; Orphan drug	TBD
roxadustat	AstraZeneca	Anemia related to CKD	Oral	Phase 3 - NDA	TBD
rucaparib (Rubraca)	Clovis	Prostate cancer	Oral	Phase 3 - sNDA	TBD
sacubitril/ valsartan (Entresto®)	Novartis	Heart failure (with preserved ejection fraction)	Oral	Phase 3 - sNDA; Fast track	TBD
sarecycline	Allergan	Acne	Oral	Phase 3 - NDA	TBD
savolitinib	AstraZeneca	RCC	Oral	Phase 3 - NDA	TBD
selinexor	Karyopharm	Multiple myeloma	Oral	Phase 3 - NDA; Orphan drug	TBD
selumetinib	AstraZeneca	Thyroid cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
seviprotimut	Polynoma	Melanoma	Intradermal	Phase 3 - BLA	TBD
siponimod	Novartis	MS (secondary progressive)	Oral	Phase 3 - NDA	TBD
somatropin	LG Life Sciences	Growth hormone deficiency; Prader-Willi syndrome	SC	Phase 3 - BLA	TBD
somavaratan	Versartis	Growth hormone deficiency	SC	Phase 3 - BLA; Orphan drug	TBD
sotagliflozin	Sanofi	T1DM; T2DM	Oral	Phase 3 - NDA	TBD
squalamine lactate	Ohr	Wet AMD	Ophthalmic	Phase 3 - NDA; Fast track	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
stannsoporfin	Infacare	Hyperbilirubinemia	IM	Phase 3 - NDA; Fast track	TBD
tafenoquine succinate	GlaxoSmithKline	Malaria	Oral	Phase 3 - NDA; Breakthrough therapy; Orphan drug	TBD
talazoparib	Pfizer	Breast cancer	Oral	Phase 3 - NDA	TBD
taselisib	Roche	Breast cancer	Oral	Phase 3 - NDA	TBD
tecarfarin	Armetheon	Anticoagulation	Oral	Phase 3 - NDA	TBD
tedopi	OSE Immunotherapeutics	NSCLC	SC	Phase 3 - NDA; Orphan drug	TBD
telatinib	Eddingpharm	Gastric cancer	Oral	Phase 3 - NDA	TBD
tenapanor	Ardelyx	IBS; Hyperphosphatemia	Oral	Phase 3 - NDA	TBD
tergenpantucel-L	Newlink Genetics	NSCLC	Intradermal	Phase 3 - BLA	TBD
teriparatide recombinant human (follow-on to Eli Lilly's Forteo®)	Pfenex	Osteoporosis; Osteopenia	SC	Phase 3 - BLA	TBD
timapiprant	Chiesi	Asthma	Oral	Phase 3 - NDA	TBD
tivozanib	AVEO	RCC	Oral	Phase 3 - NDA	TBD
tocilizumab (Actemra®)	Genentech	Scleroderma	SC	Phase 3 - sBLA; Breakthrough therapy	TBD
tosedostat	CTI	AML; Biliary tract cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
tozadenant	Acorda	Parkinson's disease	Oral	Phase 3 - NDA	TBD
tralokinumab	AstraZeneca	Asthma (severe, uncontrolled); Atopic dermatitis	SC	Phase 3 - BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Pfizer	Breast cancer (HER2+); Gastric and gastroesophageal cancer (HER2+)	IV	Phase 3 - BLA	TBD
trastuzumab (biosimilar to Genentech's Herceptin)	Samsung Bioepis	Breast cancer (HER2+); Gastric and gastroesophageal cancer (HER2+)	IV	Phase 3 - BLA	TBD
trifluridine/ tipiracil (Lonsurf®)	Otsuka	Gastric cancer	Oral	Phase 3 - sNDA	TBD
trigriluzole	Portage	Spinocerebellar ataxia	Oral	Phase 3 - NDA; Fast track; Orphan drug	TBD
triptorelin pamoate (Trelstar®)	Allergan	Breast cancer	IM	Phase 3 - sNDA	TBD
turoctocog alfa pegol	Novo Nordisk	Hemophilia A	IV	Phase 3 - BLA	TBD
ublituximab	TG	CLL/SLL	IV	Phase 3 - BLA; Orphan drug	TBD
upadacitinib	Abbvie	RA; PsA	Oral	Phase 3 - NDA	TBD

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
vadadustat	Akebia	Anemia related to CKD	Oral	Phase 3 - NDA	TBD
varicella-zoster vaccine (inactivated)	Merck	Shingles prevention	SC	Phase 3 - BLA	TBD
varlitinib	Aslan	Biliary tract cancer	Oral	Phase 3 - NDA; Orphan drug	TBD
venetoclax (Venclexta®)	Abbvie	AML; Multiple myeloma	Oral	Phase 3 - sNDA; Breakthrough therapy; Orphan drug	TBD
viaskin peanut	DBV	Peanut allergy	Transdermal	Phase 3 - BLA; Breakthrough therapy; Fast track	TBD
vilanterol trifenatate	GlaxoSmithKline	COPD; Asthma	Inhaled	Phase 3 - NDA	TBD
vinflunine ditartrate	Pierre Fabre Medicament	SCCHN	IV	Phase 3 - NDA	TBD
vocimagene amiretrorepvec	Tocagen	Anaplastic astrocytoma; Glioblastoma	Intratumoral	Phase 3 - BLA; Breakthrough therapy; Fast track	TBD
voclosporin	ILJIN	Lupus nephritis	Oral	Phase 3 - NDA; Fast track	TBD
von Willebrand factor (human; concentrate)	LFB	von Willebrand disease	IV	Phase 3 - BLA; Orphan drug	TBD
vonapanitase	Proteon	End stage renal disease (prevention of arteriovenous fistula/graft failure)	IV	Phase 3 - BLA; Breakthrough therapy; Fast track; Orphan drug	TBD
vosoritide	Proteon	Achondroplasia	SC	Phase 3 - NDA; Orphan drug	TBD
zolmitriptan microneedle patch	Zosano	Migraine	Transdermal	Phase 3 - NDA	TBD

Complete Response Letter (CRL) / Withdrawn Drugs

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
allantoin	Amicus	Epidermolysis bullosa	Topical	Withdrawn	N/A
anacetrapib	Merck	Dyslipidemia/ hypercholesterolemia	Oral	Withdrawn	N/A
CCP-07	Vernalis	Viral Rhinitis (Common cold)	Oral	CRL	TBD
CCP-08	Vernalis	Viral Rhinitis (Common cold)	Oral	CRL	TBD
dexamethasone punctum plug	Ocular	Ocular pain/inflammation	Intraocular	CRL	TBD
exenatide minipump	Intarcia	T2DM	SC	CRL	TBD
latanoprostene bunod	Valeant	Glaucoma; Ocular hypertension	Ophthalmic	CRL	TBD
nabiximol spray	Otsuka	Cancer pain	Oral	Withdrawn	N/A

PIPELINE DRUG LIST continued

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
oxycodone	Intellipharmaceutics	Chronic pain	Oral	CRL	TBD
pegfilgrastim (biosimilar to Amgen's Neulasta)	Biocon/ Mylan	Neutropenia/ leukopenia	SC	CRL	TBD
romosozumab	Amgen	Osteoporosis; Osteopenia	SC	CRL	TBD
sirukumab	Johnson & Johnson	RA	SC	CRL	TBD
sufentanil	Acelrx	Moderate to severe pain	SL	CRL	TBD

GLOSSARY

ADHD Attention Deficit Hyperactivity Disorder

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

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

DLBCL Diffuse Large B Cell Lymphoma

FDA Food and Drug Administration

ER Extended-release

GI Gastrointestinal

GLP-1 Glucagon-like peptide-1

H Half

HAP Hospital Aquired Pneumonia

HCC Hepatocellular Carcinoma

HCP Healthcare Professional

HCV Hepatitis C Virus

HIT Heparin Induced Thrombocytopenia

HTN Hypertension

IBS Irritable Bowel Syndrome

IM Intramuscular

IV Intravenous

JIA Juvenile Idiopathic Arthritis

LDL-C Low-Density Lipoprotein Cholesterol

MDD Major Depressive Disorder

MS Multiple Sclerosis

N/A Not Applicable

NDA New Drug Application

NHL Non-Hodgkin Lymphoma

NSCLC Non-Small Cell Lung Cancer

PCI Percutaneous Coronary Intervention

PDUFA Prescription Drug User Fee Application

PsA Psoriatic Arthritis

PSO Plaque Psoriasis

PTCA Percutaneous Transluminal Coronary Angioplasty

Q Quarter

RA Rheumatoid Arthritis

RCC Renal Cell Carcinoma

SL Sublingual

sBLA supplemental Biologics License Application

SC Subcutaneous

SCCHN Squamous Cell Cancer of the Head and Neck

SCLC Small Cell Lung Cancer

SLE Systemic Lupus Erythematosus

SLL Small Lymphocytic Lymphoma

sNDA supplemental New Drug Application

SSSI Skin and Skin Structure Infection

T1DM Type 1 Diabetes Mellitus

T2DM Type 2 Diabetes Mellitus

TBD To Be Determined

UA Unstable Angina

UC Ulcerative Colitis

US United States

UTI Urinary Tract Infection

XR Extended-release

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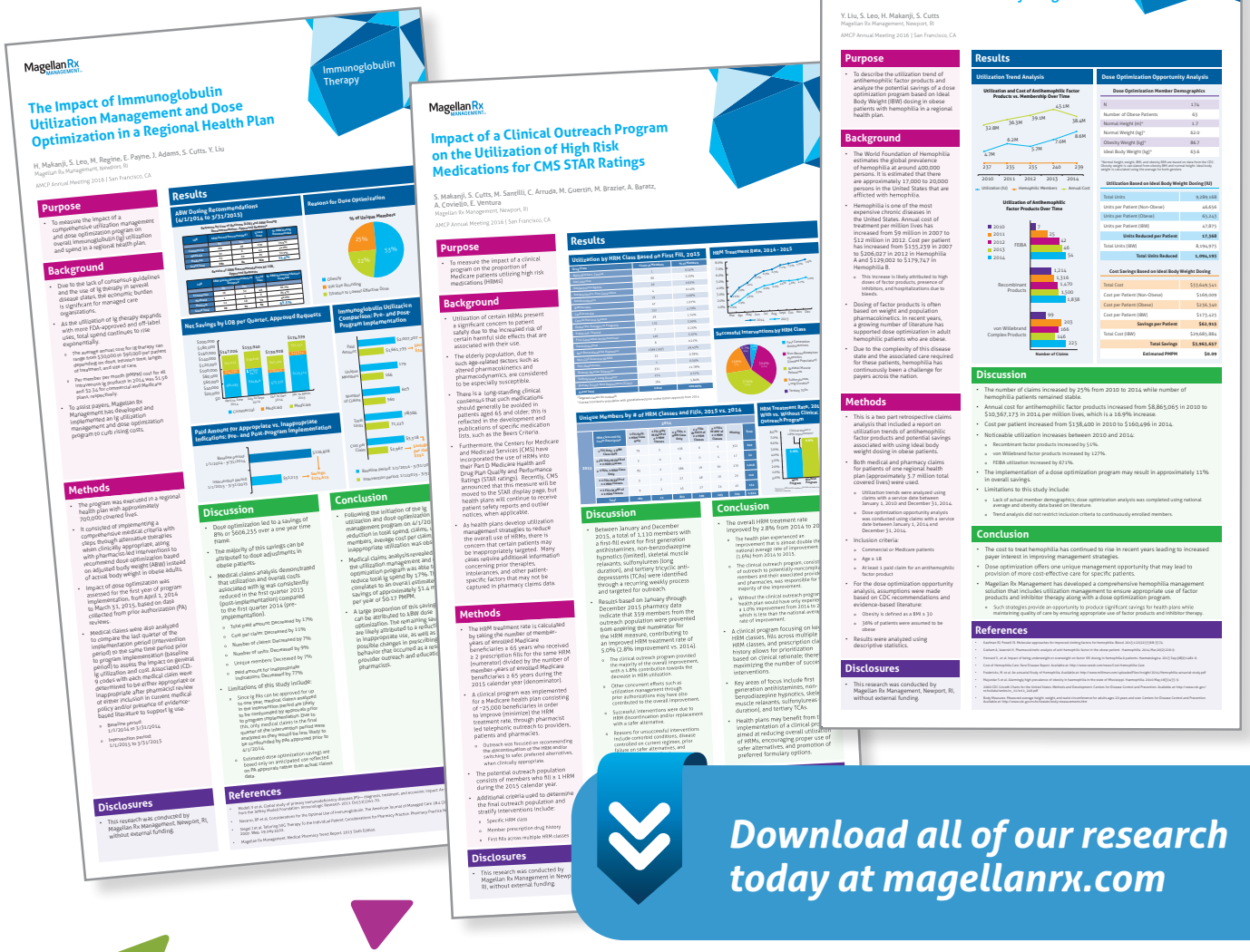

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