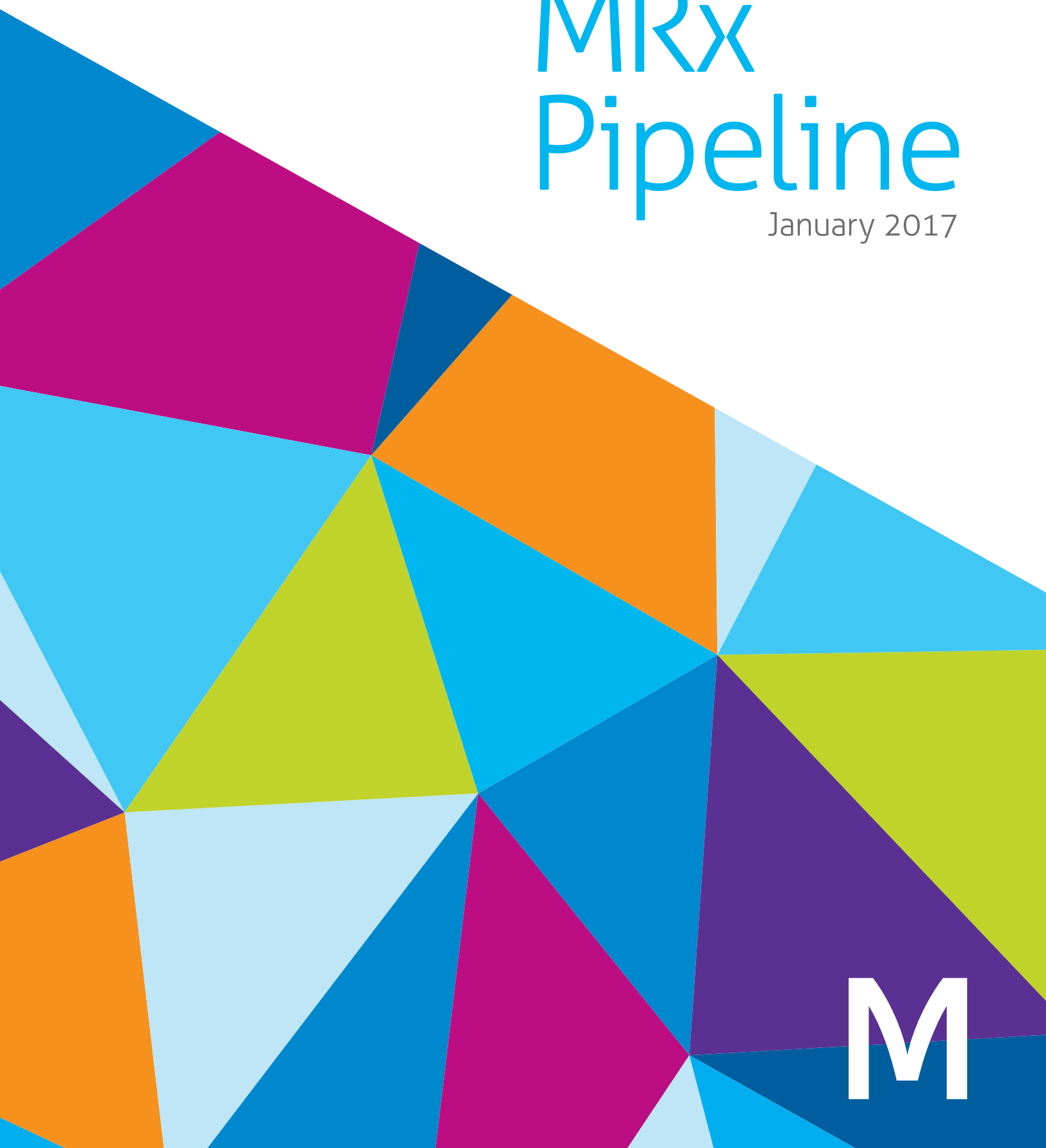
Magellan Rx MANAGEMENT℠

MRx Pipeline

January 2017



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INTRODUCTION

Welcome to the inaugural Magellan Rx Management Pipeline. Our quarterly report offers clinical insights and competitive intelligence on anticipated drugs in the pipeline. This universal forecast addresses trends applicable across market segments.

Traditional and specialty drugs in high-impact classes, agents under the pharmacy and medical benefits, new molecular entities, pertinent new and expanded indications for existing medications, and biosimilars are profiled in this unique publication.

Clinical analyses, pre-regulatory status, and financial outlook are considered as part of the multi-faceted evaluation process. The products housed in this report 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 consensus forecast data from Evaluate™, this 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.

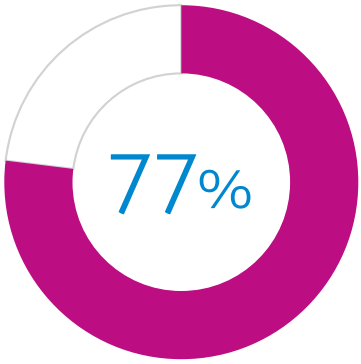
In the past few years, game changers such as products in the hepatitis C and oncology fields have blazed the pipeline trail. In 2017, a continued key trend toward the approval of specialty medications is expected. Noteworthy pipeline trends to watch in 2017 include the development of complex therapies, rare diseases, biosimilars, and the entry of genomics in the precision medicine space.

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.

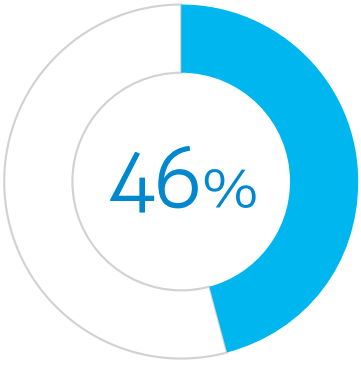
Pipeline Deep Dive

Objective evidence-based methodology was used to identify the Deep Dive drugs in the upcoming quarters that are expected to have substantial clinical impact. 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.

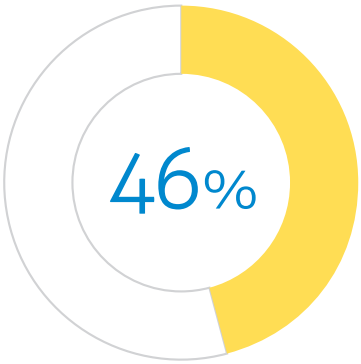
SPECIALTY



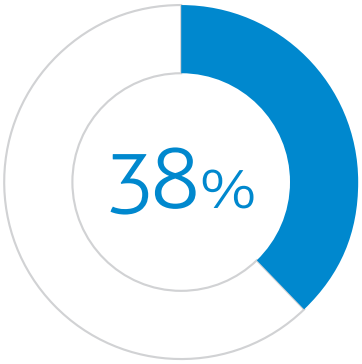
PRIORITY REVIEW



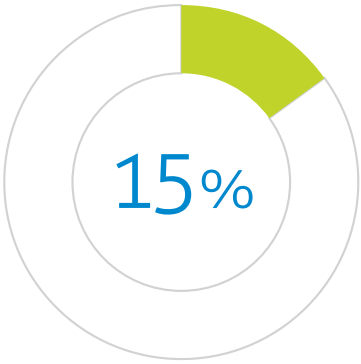
BREAKTHROUGH THERAPY



ORPHAN DRUG



BIOSIMILAR



■ Specialty drug names appear in magenta throughout the publication.

Oncology

avelumab *IV*

EMD Serono / Pfizer

PROPOSED INDICATIONS

Metastatic Merkel cell carcinoma (MCC)

CLINICAL OVERVIEW

MCC is a very rare, aggressive cutaneous neuroendocrine tumor, diagnosed most often in the elderly, immunocompromised patients, and in Caucasian males.

Avelumab is a monoclonal antibody that blocks programmed death-ligand 1 (PD-L1), a protein expressed on tumor cells and tumor-infiltrating T-cells, leading to anti-tumor immune responses. Merkel cell tumors may overexpress PD-L1 and may be caused by the Merkel cell polyomavirus integration into healthy cell DNA. Avelumab may target both mechanisms.

The pivotal phase 2 JAVELIN Merkel 200 study evaluated avelumab in 88 adults with metastatic MCC that had progressed after at least 1 chemotherapy treatment. After a median follow-up of 10.4 months, 8 patients (9.1%) achieved a complete response and 20 (22.7%) patients experienced a partial response. Ongoing responses were reported in 23 of these 28 patients. Progression-free survival (PFS) was 40% at 6 months. Presence of PD-L1 expression or Merkel cell polyomavirus did not influence response. The most common treatment-related adverse effects were fatigue (23%) and infusion-related reactions (17%); both of which were reported as grades 1 or 2 reactions.

Avelumab was studied at a dose of 10 mg/kg IV over 1 hour every 2 weeks until therapeutic failure, significant clinical deterioration, unacceptable toxicity, or other criteria indicating discontinuation.

PLACE IN THERAPY

Though still rare, the incidence of MCC is rising in the US, with approximately 1,500 new cases diagnosed each year. Overall 5-year survival is estimated at 60%; but prognosis is poorer with metastatic disease (estimated 20% at 5 years).

There is no FDA-approved treatment for MCC. Current recommended therapy for metastatic disease includes surgery, radiation therapy, and chemotherapy. Data are lacking showing that chemotherapy improves relapse-free or overall survival (OS) in patients with metastatic disease. If approved, avelumab will be the first agent indicated for the treatment of metastatic MCC, providing a more durable response compared to currently used cytotoxic agents. Avelumab is also being studied in a variety of other cancers.

FDA APPROVAL TIMELINE

May, 2017

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

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 14	$ 48	$ 131	$ 218	$ 289

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

Magellan Rx MANAGEMENT

Immunology

baricitinib *oral*

Eli Lilly / Incyte

PROPOSED INDICATIONS

Moderately to severely active rheumatoid arthritis (RA)

CLINICAL OVERVIEW

Baricitinib is a selective janus kinase (JAK) inhibitor, demonstrating greatest potency against JAK1 and JAK2. JAKs are enzymes that transmit signals within cells that affect cellular immune responses, including inflammation.

Baricitinib's clinical study program included 4 pivotal phase 3 trials in patients with moderately to severely active RA who were disease modifying antirheumatoid drug (DMARD)-naïve or inadequate responders to methotrexate (MTX), conventional DMARDs, or biologic DMARDs. Improvements in RA symptoms were reported as early as 1 week after starting baricitinib therapy. After 12 weeks, baricitinib was superior to adalimumab (Humira®; Abbvie) in patients on background MTX regarding proportion of patients that achieved a 20% symptom improvement (as measured by the American College of Rheumatology [ACR20] score) and improvements in disease activity and function (per the 28-joint Disease Activity Score [DAS28-CRP]). In addition, prevention of progression of structural joint damage, as seen on radiographic exams, was demonstrated with baricitinib as compared to placebo. Incidence of serious adverse events was similar for baricitinib compared with placebo and lower for adalimumab; rate of serious infection was similar across all groups.

Baricitinib was studied at oral doses of 2 mg and 4 mg once-daily.

PLACE IN THERAPY

An estimated 1.5 million adults in the US suffer from RA. Standard of care includes first-line use of a DMARD. If disease severity remains moderate or high, another DMARD, an anti-TNF agent, or a non-TNF biologic (all with or without MTX) should be added.

Pfizer's tofacitinib (twice-daily Xeljanz®, once-daily Xeljanz® XR) is an oral JAK inhibitor that is FDA-approved as mono- or combination therapy to treat moderately to severely active RA in adults with inadequate response or intolerance to MTX. Baricitinib may offer another option in treating RA through the JAK pathway. While baricitinib is more selective in JAK inhibition than tofacitinib, the clinical significance of this difference remains to be seen.

Other oral JAK inhibitors being studied for RA include Abbvie's upadacinitib, Astellas/Janssen's peficitinib, Gilead/Galapagos's filgotinib, and Vertex' decernotinib; all are in phase 3 trials.

FDA APPROVAL TIMELINE

January 19, 2017

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 146	$ 280	$ 479	$ 694	$ 879

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

Oncology

binimetinib *oral*

Array Biopharma

PROPOSED INDICATIONS

NRAS-mutant advanced melanoma

CLINICAL OVERVIEW

Binimetinib is a mitogen-activated extracellular signal-regulated kinase (MEK) inhibitor. MEK is a protein that when overexpressed, aids in proliferation, differentiation, migration, survival, and angiogenesis of cancer cells.

The open-label, phase 3 NEMO study reported a median progression-free survival (PFS) of 2.8 months with binimetinib compared to 1.5 months with dacarbazine in patients with NRAS Q61-mutant melanoma (stage IIIC, IVM1a, and IVM1b) who were previously untreated or progressed on/after immunotherapy. This is a statistically significant 38% reduction of risk for progression or death. The difference in overall survival (OS) was not statistically significant (median 11 months with binimetinib versus 10.1 months with dacarbazine). Patients who derived greater benefit with binimetinib included those with metastases in at least 3 organs (hazard ratio 0.4) and those who were previously treated with immunotherapy (PFS 5.5 months versus 1.6 months).

In the NEMO study, binimetinib was given as 45 mg oral doses twice-daily.

PLACE IN THERAPY

In the US, melanoma rates more than doubled in 30 years to 22.7 per 100,000 in 2011. Patients with NRAS mutations make up between 5% and 20% of cases, for which no medications are FDA-approved.

While the MEK inhibitors trametinib (Mekinist®; Novartis) and cobimetinib (Cotellic®; Genentech) are available in the US to treat BRAF-mutant advanced melanoma, neither are indicated for NRAS-mutant forms. If approved, binimetinib will be the first drug to fill this unmet need.

FDA APPROVAL TIMELINE

June 30, 2017

✓ Orphan drug

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 52	$ 156	$ 265	$ 357	$ 390

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

Endocrine

cerliponase alfa (Brineura) *IV*

Biomarin

PROPOSED INDICATIONS

Late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease

CLINICAL OVERVIEW

CLN2 disease, a form of Batten disease, is a fatal neurodegenerative disease caused by mutations in the TPP1/CLN2 gene resulting in a deficiency of the tripeptidyl peptidase 1 (TPP1) enzyme. Lack of TPP1 causes a buildup of lysosomal storage materials in neurons resulting in neurodegeneration as characterized by motor, cognitive, and visual dysfunction. Symptom onset of language delays and seizures usually begins between 2 and 4 years of age. Most patients lose their ability to walk and talk by 6 years of age. Blindness and dementia also can occur. Life expectancy is 8 to 12 years.

Cerliponase alfa is a recombinant form of human TPP1. Open-label phase 1 and 2 clinical trials of cerliponase alfa included 24 children, 3 to 16 years old, with CLN2 disease. At 48 weeks, disease stabilization was experienced by 65% of patients treated and slowed progression in 87% of patients, based on motor and language function. In addition, magnetic resonance imaging (MRI) of the brain revealed a mean total decrease in volume of cortical grey matter of 9.7% over 48 weeks; a separate study reported 14.5% annual decrease in volume in untreated patients. Durability of effect was demonstrated out to 81 weeks. Common adverse events associated with cerliponase alfa included pyrexia (46%), hypersensitivity/seizure (33% each), and vomiting/headache (13% each). No patients discontinued treatment due to adverse effects.

Cerliponase alfa was administered as 300 mg via intracerebroventricular infusion every 14 days.

PLACE IN THERAPY

CLN2 disease is a rare degenerative disease occurring in approximately 1 in 200,000. There are no approved treatment options for this condition. Current care focuses on symptom management, prevention and treatment of complications, and quality of life. If approved, cerliponase alfa will be the first agent that may attenuate decline or stabilize the disease.

FDA APPROVAL TIMELINE

April 27, 2017

✓ Breakthrough therapy ✓ Orphan drug ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 8	$ 30	$ 53	$ 81	$ 101

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

Central nervous system

deutetrabenazine (Austedo) *oral*

Auspex / Teva

PROPOSED INDICATIONS

Chorea associated with Huntington disease

CLINICAL OVERVIEW

Deutetrabenazine inhibits vesicular monoamine 2 transporter (VMAT2), which depletes dopamine levels in the brain. It is a deuterated form of tetrabenazine (Xenazine®; Valeant) with diminished cytochrome P450 CYP2D6 metabolism allowing for a longer half-life while maintaining drug efficacy.

Chorea (abnormal involuntary movement) is a characteristic feature of Huntington disease, a rare and fatal genetic disorder resulting in neurodegeneration of the brain. As the disease progresses, chorea is replaced by dystonia and parkinsonism. In a pivotal clinical trial, treatment with deutetrabenazine was associated with statistically significant improvement in chorea (difference of change in chorea score of -2.5 points), dystonia, and physical function over placebo after 12 weeks of therapy. Patients and clinicians reported that a significantly greater proportion of patients treated with deutetrabenazine were "much" or "very much" improved. No significant differences in balance were revealed. Adverse events were similar between groups.

Deutetrabenazine was given as oral doses twice-daily. Dosages studied were based on clinical response and did not exceed 48 mg per day.

PLACE IN THERAPY

Approximately 30,000 American's have symptomatic Huntington disease; about 90% of which experience chorea at some point. There is no cure and treatment is symptomatic. While chorea may not be debilitating, as it becomes more severe, it can interfere with patients' function. Tetrabenazine may be an effective treatment; riluzole may only offer marginal improvement with off-label use. Tetrabenazine is dosed orally 3 times a day, and its use is associated with depression/suicidality, parkinsonism, anxiety, insomnia, and somnolence. Genotyping may be needed for patients since tetrabenazine is metabolized by CYP2D6. As compared to tetrabenazine, deutetrabenazine pharmacokinetics allow it to be dosed less frequently and at lower dosages; thus it may provide a better safety profile while maintaining clinical efficacy for the symptomatic treatment of chorea.

FDA APPROVAL TIMELINE

April 3, 2017

✓ Orphan drug

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 113	$ 120	$ 167	$ 212	$ 255

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

Immunology

dupilumab (Dupixent) *sc*

Regeneron / Sanofi

PROPOSED INDICATIONS

Moderate to severe atopic dermatitis (AD) in adults

CLINICAL OVERVIEW

Moderate to severe forms of AD are characterized by pronounced itching, skin dryness and lesions that may involve crusting, oozing and thickening of the skin. AD may be chronic or relapsing in nature. Other inflammatory comorbid conditions, such as asthma, may be present and the patient may have a family history of AD, asthma, or allergies.

Dupilumab is a human monoclonal antibody that inhibits interleukins-4 and -13 (IL-4, IL-13), two cytokines involved in allergic inflammatory responses moderated by Type 2 helper (Th2) T-cells.

Two pivotal phase 3 trials, SOLO-1 and SOLO-2, enrolled adults with moderate or severe AD that affected approximately 50% of their body surface area and was not controlled with topical therapy. In both studies, significantly more patients given dupilumab achieved "clear" or "almost clear" skin compared to those treated with placebo (36-38% versus 8-10%) after 16 weeks. Mild to moderate injection site reactions and conjunctivitis were reported more frequently with dupilumab than placebo.

In both trials, dupilumab was administered SC at doses of 600 mg on day 1 and 300 mg either weekly or every other week thereafter. Results were similar for both dosing regimens.

PLACE IN THERAPY

An estimated 17.8 million Americans are affected by AD, and up to 20% of cases are moderate to severe in nature, having a significant negative impact on quality of life. Topical moisturizers are an integral part of AD management. Therapy with topical corticosteroids (TCS) is added during flares and is associated with cutaneous and systemic adverse effects with long-term use. The topical calcineurin inhibitor (TCI) tacrolimus (Protopic®; Astellas and generic) is an alternative when TCS use is not appropriate; TCIs carry a boxed warning for risk of rare malignancy (e.g., skin and lymphoma).

Dupilumab may offer a systemic arsenal for adults whose AD is not controlled with topical therapy. Studies of longer duration are needed to assess its long-term efficacy and safety. Dupilumab is also being studied for uncontrolled persistent asthma.

FDA APPROVAL TIMELINE

March 29, 2017

✓ Breakthrough therapy ✓ Fast track ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 216	$ 480	$ 923	$ 1,423	$ 1,720

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

Magellan Rx MANAGEMENT℠

Central nervous system

edaravone (Radicava) *IV*

Mitsubishi Tanabe

PROPOSED INDICATIONS

Amyotrophic lateral sclerosis (ALS)

CLINICAL OVERVIEW

ALS is a progressive neurodegenerative disease affecting the brain and spinal cord and can lead to paralysis and death within 2 to 5 years from diagnosis. It is most often diagnosed between the ages of 40 and 70 years. Causes include genetic and environmental factors; 5% to 10% of cases are inherited. Oxidative stress may contribute to the onset and progression of ALS as patients with ALS have consistent increases in oxidative stress biomarkers.

Edaravone is a free radical scavenger that may decrease the effects of oxidative stress on the body. A phase 2 study reported slowed progression of motor dysfunction and low levels of the oxidative stress biomarker, 3-nitrotyrosine (3NT), in patients treated with 6 cycles of edaravone. However, a confirmatory trial in 205 patients with ALS progression, as indicated by the revised ALS functional rating scale (ALSFRS-R), did not reproduce a statistically significant slowing of progression of motor dysfunction when given the same regimen of edaravone.

In clinical studies, edaravone was administered as 60 mg IV daily for 14 days followed by 14 days without drug.

PLACE IN THERAPY

It is estimated that ALS affects 5 per 100,000 people in the US. There is no cure for ALS. Treatment includes multidisciplinary supportive measures. Medications are prescribed to reduce fatigue, muscle cramping, spasticity, and excess saliva. Oral riluzole (Rilutek®; Covis) was FDA-approved in 1995 and has been shown to delay need for ventilation support and prolong survival by reducing motor neuron damage through decreased glutamate levels.

Edaravone may be the first drug to be FDA-approved for ALS in over 20 years that may slow progression of the disease, although, data is limited to conclude a disease benefit.

Other drugs being studied for ALS include Cytokinetic's oral fast skeletal muscle troponin agent, tirasemtiv that increases skeletal muscle force and delays muscle fatigue. In addition, AB Science reported that a predefined interim analysis of their phase 2/3 study of the oral tyrosine kinase inhibitor masitinib met its primary objective in change from baseline in ALSFRS-R, when used in combination with riluzole for 48 weeks. Masitinib targets microglial and mast cells and may reduce necrosis and atrophy of motorneurons.

FDA APPROVAL TIMELINE

June 16, 2017

✓ Orphan drug

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 90	$ 123	$ 161	$ 190	$ 199

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

Endocrine

liraglutide (Victoza®) *SC*

Novo Nordisk

PROPOSED INDICATIONS

CV risk reduction in adults with T2DM at high CV risk

SC liraglutide is already approved and currently available as a pre-filled once-daily pen for the treatment of T2DM as adjunct to diet and exercise.

CLINICAL OVERVIEW

Liraglutide is a glucagon-like peptide-1 (GLP-1) receptor agonist.

The LEADER trial followed 9,340 patients with T2DM over a median of 3.8 years. It reported a significant 13% relative reduction in first occurrence of major coronary event (death from CV cause, nonfatal MI, or nonfatal stroke) in patients with T2DM who were treated with liraglutide compared to those treated with placebo. The CV benefit was mostly due to reduction in death from CV or other causes (22% and 15%, respectively); there was no significant decrease in the rates of nonfatal MI, nonfatal stroke, or hospitalization for heart failure.

Liraglutide doses up to 1.8 mg were studied.

PLACE IN THERAPY

Adults with T2DM have a higher risk of CV complications, including approximately a 1.7 times higher risk of CV-related death. If approved, liraglutide will be the second antidiabetic agent to gain a cardioprotective indication in patients with T2DM.

In December 2016, the sodium-glucose co-transporter 2 (SGLT2) inhibitor, empagliflozin (Jardiance®; Boehringer Ingelheim), received a new indication for the reduction of risk of CV death in adults with T2DM and established CVD. In a pivotal study, empagliflozin led to a 14% reduction in first occurrence of a major CV event compared to placebo. Similarly, the benefit was almost entirely due to a 38% reduction in CV death. The GLP-1 agonist lixisenatide (Adlyxin™, Sanofi) was found to have no CV risks or benefits associated with its use.

FDA APPROVAL TIMELINE

H1, 2017

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 2,413	$ 2,537	$ 2,599	$ 2,643	$ 2,650

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

Central nervous system

ocrelizumab (Ocrevus) *IV*

Genentech / Roche

PROPOSED INDICATIONS

Primary progressive multiple sclerosis (PPMS), Relapsing multiple sclerosis (RMS)

CLINICAL OVERVIEW

RMS is the more common form of multiple sclerosis (MS) and is characterized by exacerbations of neurologic symptoms followed by periods of remission. PPMS is usually a steady worsening of neurologic function without distinct relapses or remission. Ocrelizumab, a humanized monoclonal antibody that targets CD20+ B-cells, is believed to affect myelin and axonal damage in MS.

In 2 phase 3 clinical studies, OPERA I and OPERA II, ocrelizumab was associated with a significant reduction in annualized relapse rates of approximately 46% compared with thrice-weekly interferon beta-1a (Rebif®) in people with RMS. A post-hoc analysis of the data revealed that 75% more patients treated with ocrelizumab achieved no evidence of disease activity (NEDA) than patients treated with interferon beta-1a over 96 weeks.

The phase 3 ORATORIO study reported significant reductions in disability progression (by 24%), decreased volume of brain lesions (by 3.4%), and whole brain volume loss (by 17.5%) with ocrelizumab compared to placebo in patients with PPMS. A post-hoc analysis used a composite endpoint to report that 47% more patients on ocrelizumab achieved no evidence of progression (NEP) compared to placebo.

Overall, reports of adverse events were similar when comparing ocrelizumab to interferon beta-1a and placebo. The most common adverse events associated with ocrelizumab were mild to moderate infusion reactions and infections. Notably, in a phase 2 RMS trial, 1 death occurred due to systemic inflammatory response syndrome (SIRS).

In phase 3 MS clinical studies, ocrelizumab was administered as 600 mg via IV infusions every 6 months; in the studies for PPMS, this dose was divided as 2 doses of 300 mg, administered 2 weeks apart, every 6 months.

PLACE IN THERAPY

MS affects approximately 90 per 100,000 people in the US, about 15% of which have a primary progressive form. Currently, there are several oral and injectable agents with varying mechanisms of action available to decrease the frequency of exacerbations and mitigate physical disability in people with RMS. However, therapies for PPMS are lacking.

Ocrelizumab seeks to be the first effective therapy for the treatment of PPMS. Marketed disease-modifying therapies for RMS work by reducing inflammation in the central nervous system and are not as effective for PPMS, where inflammation plays a smaller role. Ocrelizumab demonstrated significantly greater reduction in relapse rates than interferon beta-1a (Rebif) in patients with RMS.

Other agents used to treat relapsing forms of MS also carry risks. Injectable natalizumab (Tysabri®; Biogen) and oral dimethyl fumarate (Tecfidera®; Biogen) and fingolimod (Gilenya®; Novartis) have been associated with progressive multifocal leukoencephalopathy (PML), which can be fatal. Daclizumab (Zinbryta™; Biogen/Abbvie) is a third-line therapy due to its safety profile. While ocrelizumab has the opportunity to fill a niche in PPMS and compete in the RMS domain, long-term safety will also determine its place in therapy.

FDA APPROVAL TIMELINE

March 28, 2017

✓ Breakthrough therapy ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 406	$ 947	$ 1,419	$ 1,774	$ 2,015

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

Magellan Rx MANAGEMENT

Oncology

ribociclib *oral*

Astex / Novartis

PROPOSED INDICATIONS

Hormone receptor positive, human epidermal growth factor receptor-2 negative (HR+/HER2-) advanced or metastatic breast cancer in postmenopausal women

CLINICAL OVERVIEW

Ribociclib is a selective cyclin dependent kinase (CDK4/6) inhibitor. It slows progression of breast cancer by inhibiting cyclin dependent kinase 4 and 6 proteins, which promote cancer cell proliferation.

The phase 3 MONALEESA-2 trial demonstrated superiority of ribociclib plus the aromatase inhibitor letrozole compared to letrozole alone as first-line therapy in postmenopausal women with HR+/HER2- advanced breast cancer (n=668). Ribociclib provided a 44% improvement in progression-free survival (PFS). Median PFS was 14.7 months for letrozole alone but was not reached in the ribociclib arm at data cut-off. The number of deaths during the study was too low to evaluate impact on overall survival. More patients treated with ribociclib experienced neutropenia, leukopenia, lymphopenia, elevated liver enzymes, nausea, infection, and diarrhea. Discontinuation rate due to adverse events was 7.5% in those treated with ribociclib.

Ribociclib was administered as daily oral doses of 600 mg (3 weeks on and 1 week off) in combination with letrozole per the approved label.

PLACE IN THERAPY

It is currently estimated that each year 300,000 women are diagnosed with breast cancer in the US, and each year an estimated 40,000 women will die from the disease. Approximately 74% of breast cancers are HR+/HER2-, also known as luminal A subtype, which is slow-growing and has a more favorable prognosis than other breast cancer subtypes (HR+/HER2+ or HR-/HER2+ or HR-/HER2-).

The first oral CDK4/6 inhibitor, palbociclib (Ibrance®; Pfizer) was FDA-approved in combination with letrozole as first-line for HR+/HER2- advanced or metastatic breast cancer in postmenopausal women. Palbociclib has a similar safety profile as ribociclib. There are no studies comparing the two CDK4/6 inhibitors. Cost of ribociclib and established market presence of palbociclib may limit ribociclib's market uptake.

Palbociclib is also FDA-approved in combination with the estrogen receptor down regulator fulvestrant (Faslodex®; AstraZeneca) in HR+/HER2- women with disease progression following endocrine therapy. Other breast cancer trials by Novartis are currently evaluating the efficacy and safety of ribociclib plus fulvestrant in men and postmenopausal women (HR+/HER2-) and in combination with goserelin in premenopausal women (HR+/HER2-).

FDA APPROVAL TIMELINE

May, 2017

✓ Breakthrough therapy ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 121	$ 374	$ 560	$ 724	$ 796

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

Magellan Rx MANAGEMENT

Central nervous system

valbenazine (Ingrezza) *oral*

Neurocrine Biosciences

PROPOSED INDICATIONS

Tardive dyskinesia (TD)

CLINICAL OVERVIEW

TD, involuntary movements of the tongue, lips, face, trunk, and extremities, may occur in patients treated with dopamine receptor blockers, such as antipsychotic agents. Valbenazine is a highly selective vesicular monoamine transporter 2 (VMAT2) inhibitor that modulates the release of dopamine during nerve communication.

The efficacy of valbenazine, a purified (+) alpha isomer of tetrabenazine, was studied in the KINECT 3 trial in 234 patients with moderate to severe TD with underlying schizophrenia, schizoaffective disorder, or mood disorder (bipolar disorder or major depressive disorder). Valbenazine 80 mg was associated with a significantly greater improvement in TD compared to placebo, as measured by the Abnormal Involuntary Movement Scale (AIMS). Valbenazine 40 mg also resulted in significant change in AIMS, but this dose was not included in the primary outcome measure. Valbenazine was well tolerated. No increased risk of psychiatric symptoms, including suicidality, were evident. The majority of patients received at least 1 concomitant antipsychotic medication; no drug interactions between valbenazine and antipsychotics were idenitified. The most common adverse effect reported was somnolence; incidence was similar for valbenazine and placebo.

Valbenazine is dosed orally once-daily.

PLACE IN THERAPY

There are currently no FDA-approved drugs for the treatment of TD, which is typically irreversible. Use of a neuroleptic agent for the shortest duration is recommended to prevent TD from occurring. Data are lacking to recommend for or against withdrawal of causative agents to treat TD. Antipsychotic agents with a lower TD risk are available. The American Academy of Neurology recommends consideration of clonazepam and ginkgo biloba for TD; amantadine and tetrabenazine may be considered.

Valbenazine may be the first drug approved to treat TD for patients who also require treatment with a dopamine receptor blocker.

FDA APPROVAL TIMELINE

April 11, 2017

✓ Breakthrough therapy ✓ Priority review

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 40	$ 149	$ 319	$ 626	$ 953

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 any meaningful clinical differences.

Many controversies surround biosimilars. The FDA has issued final and draft guidances, but regulatory hurdles remain. There is currently not a uniform non-proprietary naming convention. The international nonproprietary name (INN) impacts interchangeability as it affects the pharmacists' ability to substitute an interchangeable biosimilar for the reference product. 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. But, 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 to be biologics by the FDA, any generics are technically branded competitors and are not considered biosimilars under FDA's definition. However, in practice, 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 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. Sandoz' Zarxio® filgrastim-sndz and Pfizer's Inflectra™ infliximab-dyyb are currently the only FDA-approved biosimilars which have entered the market.

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. However, this potential cost-savings can vary based on the market segment, where brand contracts can play a role.

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 difficult to treat diseases, while reducing costs.

Endocrine

insulin glargine (MK-1293) *injectable*

Merck / Samsung Bioepis

MK-1293 is a follow-on insulin to Sanofi's Lantus®, indicated for the treatment of type 1 and type 2 diabetes mellitus. MK-1293 has the same amino acid sequence as the originator product. Clinical trials show it to be equivalent regarding effectiveness, safety, and immunogenicity to Lantus.

Eli Lilly started marketing Basaglar®, their follow-on product to Lantus, in December 2016.

FDA APPROVAL TIMELINE

2017

FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 2,741	$ 1,969	$ 1,667	$ 1,438	$ 1,283

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

Immunology

infliximab (SB2) *injectable*

Merck / Samsung Bioepis

SB2 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 SB2 to Remicade in patients with moderate to severe RA.

Pfizer launched infliximab-dyyb (Inflectra™), their biosimilar to Remicade, in late November 2016. Amgen also has a biosimilar version of Remicade in phase 3 clinical trials.

FDA APPROVAL TIMELINE

January, 2017

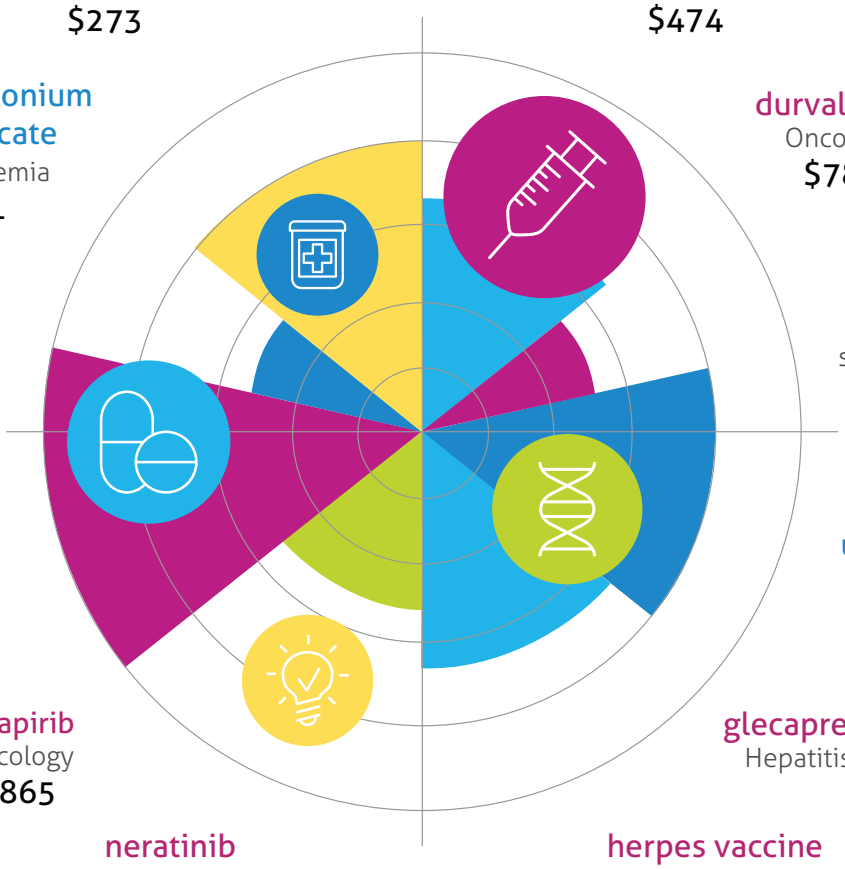
FINANCIAL FORECAST (reported in millions)

2017	2018	2019	2020	2021
$ 4,767	$ 4,464	$ 3,761	$ 3,257	$ 2,895

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 innovative 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 proposed clinical use, as well as an estimated financial forecast for the year 2021 are displayed. The financials represent projected total annual US sales, reported in millions.



anifrolumab
Systemic lupus erythematosus
$97

bio-identical 17-beta-estradiol
Womens health
$474

durvalumab
Oncology
$781

elafibranor
Non alcoholic steatohepatitis (NASH)
$974

fluticasone furoate/ umeclidinium bromide/ vilanterol
COPD
$336

glecaprevir/ pibrentasvir
Hepatitis C - pangenotypic
$329

herpes vaccine
Shingles vaccine
$502

naldemedine tosylate
Opioid-induced constipation
$523

neratinib
Oncology
$618

nirapirib
Oncology
$865

plecanatide
Chronic idiopathic constipation
$517

sirukumab
RA
$432

sodium zirconium cyclosilicate
Hyperkalemia
$451

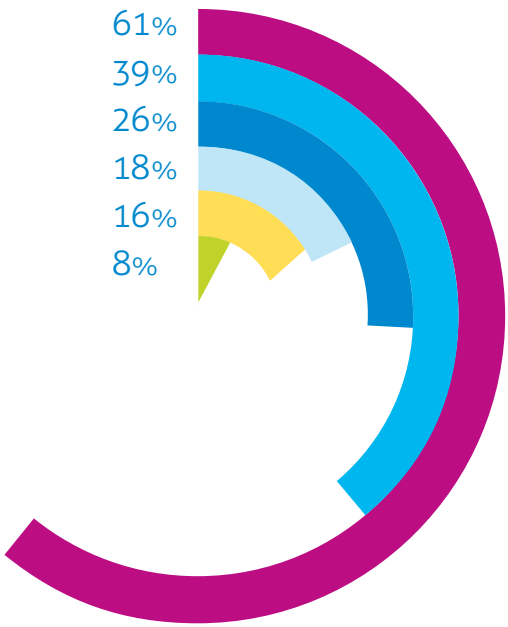
sofosbuvir/ velpatasvir/ voxilaprevir
Hepatitis C - pangenotypic
$273

Specialty drug names appear in magenta throughout the publication.

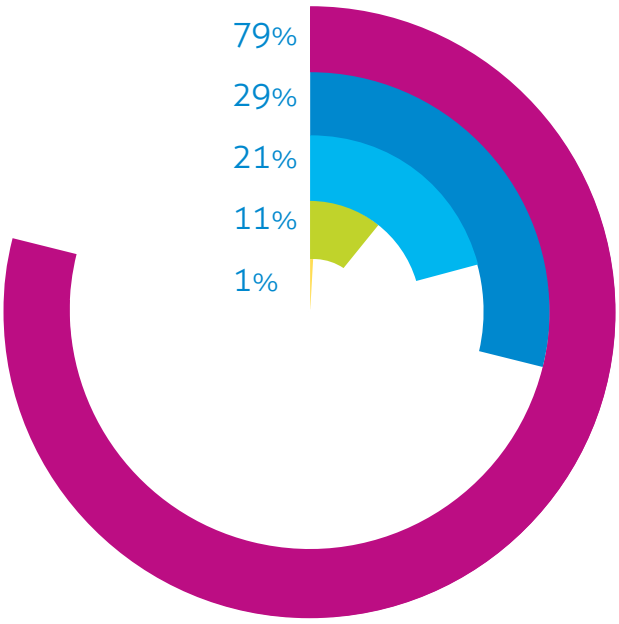
Pipeline Drug List

The Pipeline Drug List paints 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

PIPELINE DRUG LIST

Specialty drug names appear in magenta.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
linaclotide (Linzess®) 72 mcg	Allergan	Chronic idiopathic constipation (CIC)	Oral	Submitted	Early 2017
aripiprazole lauroxil (Aristada®) - every 2 month dosing	Alkermes	Schizophrenia - 2-month dosing interval	IM	Submitted	2017
CSL830	CSL Behring	Hereditary angioedema prophylaxis	SC	Submitted	2017
glycerol phenylbutyrate (Ravicti)	Horizon	Urea cycle disorders (ages 2 months to 2 years)	Oral	Submitted; Orphan drug	2017
hiv-1 immunogen vaccine (Remune)	Immune Response Biopharma	HIV infection	IM	Submitted; Orphan drug (pediatrics)	2017
insulin glargine (follow-on of Sanofi-Aventis' Lantus®)	Merck/ Samsung Bioepis	T1DM; T2DM	SC	Submitted	2017
liraglutide (Victoza®)	Novo Nordisk	Reduction in major adverse CV events in patients with T2DM	SC	Submitted	2017
fluticasone/ salmeterol MDPI	Teva	Asthma	Inhalation	Submitted	Q1-2017
fluticasone propionate RespiClick	Teva	Asthma	Inhalation	Submitted	Q1-2017
oxymetazoline	Allergan	Persistent facial erythema of rosacea	Topical	Submitted	H1-2017
infliximab (biosimilar to Janssen's Remicade®)	Merck/ Samsung Bioepis	RA; CD; UC; AS; PSA; Plaque psoriasis	SC	Submitted	January, 2017
rolapitant (Varubi®)	OPKO/ Tesaro	Chemotherapy induced nausea and vomiting (CINV)	IV	Submitted	1/11/2017
baricitinib	Eli Lilly/ Incyte	RA	Oral	Submitted	1/19/2017
plecanatide	Synergy	Chronic idiopathic constipation (CIC)	Oral	Submitted	1/29/2017
acetaminophen/ hydrocodone/ promethazine	Charlestone Labs/ Daiichi Sankyo	Moderate to severe acute pain with prevention of opioid induced nausea and vomiting (OINV)	Oral	Submitted	1/31/2017

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
deflazacort	Marathon	Duchenne muscular dystrophy	Oral	Submitted; Fast track; Orphan drug; Priority review; Rare pediatric disease status	February, 2017
MK-8237	ALK-Abello/ Merck	Allergic rhinitis (house dust mite induced) - immunotherapy	Sublingual	Submitted	February, 2017
brodalumab (Siliq)	AstraZeneca/ Valeant	Plaque psoriasis	SC	Submitted	2/16/2017
daratumumab (Darzalex®)	Genmab/ Janssen	Multiple myeloma	IV	Submitted; Breakthrough therapy; Orphan drug; Priority review	2/17/2017
telotristat etiprate	Ipsen/ Lexicon	Carcinoid syndrome	Oral	Submitted; Breakthrough therapy; Fast track; Orphan drug; Priority review	2/28/2017
coagulation factor IX (recombinant)	Novo Nordisk	Hemophilia B	IV	Submitted	March-April, 2017
pembrolizumab (Keytruda®)	Merck	Hodgkin's lymphoma	IV	Submitted; Breakthrough therapy; Priority review	3/15/2017
safinamide (Xadago)	Meiji Seika/ Newron	Parkinson's disease	Oral	Submitted	3/21/2017
naldemedine tosylate	Shionogi	Opioid-induced constipation	Oral	Submitted	3/23/2017
ocrelizumab (Ocrevus)	Genentech/ Roche	Primary progressive MS; Relapsing MS	IV	Submitted; Breakthrough therapy; Priority review	3/28/2017
dupilumab (Dupixent)	Regeneron/ Sanofi	Atopic dermatitis	SC	Submitted; Breakthrough therapy; Fast track; Priority review	3/29/2017
abaloparatide	Radius Health	Postmenopausal osteoporosis	SC	Submitted	3/30/2017

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
delafloxacin meglumine (Baxdela)	Abbvie/ Ligand/ Melinta	Hospital-treated acute bacterial skin and skin structure infections (ABSSSI)	Oral, IV	Submitted; Qualified infectious disease product	Q2-2017
durvalumab	AstraZeneca/ Medimmune	Bladder cancer; NSCLC	IV	Submitted	Q2-2017
lisdexamfetamine chewable (Vyvanse®)	Shire	ADHD; Binge eating disorder	Oral	Submitted	Q2-2017
ozenoxacin	Ferrer/ Medimetriks	Impetigo	Topical	Submitted	Q2-2017
sodium zirconium cyclosilicate	AstraZeneca/ ZS	Hyperkalemia	Oral	Submitted	April, 2017
deutetrabenazine (Austedo)	Auspex/ Teva	Chorea associated with Huntington's disease	Oral	Submitted; Orphan drug	4/03/2017
valbenazine (Ingrezza)	Neurocrine	Tardive dyskinesia	Oral	Submitted; Breakthrough therapy; Priority review	4/11/2017
cerliponase alfa (Brineura)	Biomarin	CLN2 disease	IV	Submitted; Breakthrough therapy; Orphan drug; Priority review	4/27/2017
brigatinib	Ariad	NSCLC	Oral	Submitted; Orphan drug	4/29/2017
atezolizumab (Tecentriq®)	Genentech/ Roche	Advanced/metastatic urothelial cancer	IV	Submitted; Priority review	4/30/2017
avelumab	EMD Serono/ Pfizer	Merkel cell carcinoma	IV	Submitted; Breakthrough therapy; Fast track; Orphan drug; Priority review	May, 2017
midostaurin	Novartis	AML (adults with FLT3 mutation); Advanced mastocytosis	Oral	Submitted; Breakthrough therapy; Orphan drug; Priority review	May, 2017
ribociclib	Astex/ Novartis	Breast cancer (HER2-positive)	Oral	Submitted; Breakthrough therapy; Priority review	May, 2017

Magellan Rx MANAGEMENT

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
bio-identical 17-beta-estradiol (Yuvvexy)	Therapeutics MD	Moderate-severe vaginal pain during sexual intercourse (dyspareunia)	Vaginal	Submitted	5/07/2017
glycopyrronium bromide	Sumitomo Dainippon/ Sunovion	COPD	Inhalation	Submitted	5/29/2017
pegfilgrastim (biosimilar to Amgen's Neulasta®)	Coherus	Neutropenia associated with myelosuppressive chemotherapy	SC	Submitted	6/09/2017
edaravone (Radicava)	Jiangsu Simcere Mitsubishi Tanabe	Amyotrophic lateral sclerosis	IV	Submitted; Orphan drug	6/16/2017
betrixaban	Portola	Venous thromboembolism	Oral	Submitted; Priority review	6/24/2017
binimetinib	Array Biopharma	Melanoma	Oral	Submitted; Orphan drug	6/30/2017
niraparib	Janssen/ Tesaro	Recurrent ovarian, fallopian tube, or peritoneal cancers	Oral	Submitted; Orphan drug (ovarian cancer); Priority review	6/30/2017
allopurinol/ lesinurad (Duzallo)	Ironwood	Hyperuricemia associated with uncontrolled gout	Oral	Submitted; Orphan drug	Q3-2017
belimumab (Benlysta SC)	GlaxoSmithKline	Systemic lupus erythematosus (active autoantibody-positive)	SC	Submitted	Q3-2017
ibrutinib (Imbruvica®)	Janssen/ Pharmacyclics	Marginal zone lymphoma (MZL)	Oral	Submitted; Orphan drug	Q3-2017
perampanel (Fycompa®)	Eisai	Partial onset seizures (monotherapy)	Oral	Submitted	Q3-2017
rabies immune globulin, human (KedRAB)	Kamada	Post-exposure treatment of rabies	IM	Submitted	Q3-2017
sirukumab	GlaxoSmithKline/ Janssen	RA	SC	Submitted	Q3-2017
neratinib	Puma Biotechnology	Breast cancer (HER2-positive)	Oral	Submitted	July, 2017
regorafenib (Stivarga®)	Bayer	Hepatocellular carcinoma (second-line)	Oral	Submitted; Fast track; Priority review	July, 2017
L-glutamine, pharmaceutical grade	Emmaus	Sickle cell disease	Oral	Submitted; Fast track; Orphan drug	7/07/2017

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
romosozumab	Amgen/ UCB	Postmenopausal osteoporosis	SC	Submitted	7/19/2017
aripiprazole (Abilify Maintena®)	Bristol-Myers Squibb/ Otsuka	Bipolar I (maintenance)	IM	Submitted	7/28/2017
amantadine ER	Adamas	Drug-induced dyskinesia associasted with Parkinson's disease	Oral	Submitted; Orphan drug	8/24/2017
rituximab (Rituxan®)	Genentech/ Roche	Diffuse large B cell lymphoma; Follicular lymphoma	SC	Submitted; Orphan drug	September, 2017
trastuzumab (biosimilar to Genentech's Herceptin®)	Biocon/ Mylan	Breast cancer (HER2-positive); Gastric cancer; Gastroesophageal junction cancer	IV	Submitted; Orphan drug	September, 2017
amphetamine XR oral solution	Neos Therapeutics	ADHD	Oral	Submitted	Q4-2017
axicabtagene ciloleuce	Kite	ALL; B-cell lymphoma; Diffuse large B-cell lymphoma; Follicular lymphoma; Mantle cell lymphoma	IV	Submitted	Q4-2017
cytarabine/ daunorubicin (Vyxeos)	Celator	AML	IV	Submitted	Q4-2017
dantrolene (Ryanodex®)	Eagle	Exertional heat stroke	IV	Submitted	Q4-2017
exenatide (continuous delivery)	Intarcia Therapeutics	T1DM; T2DM	Implant	Submitted	Q4-2017
fluticasone furoate/ umeclidinium bromide/ vilanterol trifenatate	GlaxoSmithKline/ Theravance	COPD	Inhalation	Submitted	Q4-2017
glecaprevir/ pibrentasvir	Abbvie	Hepatitis C genotypes 1-6	Oral	Submitted; Breakthrough therapy	Q4-2017
golimumab (Simponi Aria®)	Janssen	AS; PsA	SC	Submitted	Q4-2017
guselkumab	Janssen	Chronic plaque psoriasis; Pustular psoriasis	SC	Submitted	Q4-2017
herpes vaccine (Shingrix)	GlaxoSmithKline	Herpes zoster (shingles) vaccine	IM	Submitted	Q4-2017

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
sofosbuvir/ velpatasvir/ voxilaprevir	Gilead	Hepatitis C genotypes 1-6	Oral	Submitted; Breakthrough therapy	Q4-2017
voretigene neparvovec	Spark Therapeutics	Inherited retinal disease	Ophthalmic	Submitted; Breakthrough therapy; Orphan drug	Q4-2017
daptomycin	Sagent	Complicated skin and skin structure infections (CSSSI); Staphylococcus septicemia	IV	Submitted	Pending
filgrastim (Grastofil; biosimilar to Amgen's Neupogen)	Accord/ Apotex/ Intas	Cancer patients receiving bone marrow transplant; Cancer patients receiving myelosuppressive chemotherapy; Severe chronic neutropenia	SC, IV	Submitted	Pending
hydrocodone ER abuse-deterrent (Vantrela ER)	Teva	Moderate to severe chronic pain	Oral	Submitted	Pending
netarsudil (Rhopressa)	Aerie	Open-angle glaucoma; Ocular hypertension	Ophthalmic	Submitted	Pending
pegfilgrastim (Lapelga; biosimilar to Amgen's Neulasta®)	Accord/ Apotex/ Intas	Cancer patients receiving myelosuppressive chemotherapy	SC	Submitted	Pending
semaglutide	Novo Nordisk	T1DM; T2DM	SC	Submitted	Pending
ustekinumab SC (Stelara® SC)	Janssen	Psoriasis (ages 12-17 years)	SC	Submitted	Pending
abatacept (Orencia®)	Bristol-Myers Squibb	Lupus nephritis; Psoriatic arthritis; Sjogren's syndrome;	SC	Phase 3	TBD
abemaciclib	Eli Lilly	Breast cancer (HER2-positive); NSCLC	Oral	Phase 3; Breakthrough therapy	TBD
abicipar pegol	Allergan/ Actavis/ Molecular Partners	Wet age-related macular degeneration	Intravitreal	Phase 3	TBD
abiraterone acetate (Yonza)	Churchill/ iCeutica	Prostate cancer	Oral	Phase 3	TBD
acalabrutinib	Acerta/ AstraZeneca/ Merck	CLL; Mantle cell lymphoma	Oral	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
adalimumab (biosimilar to Abbvie's Humira®)	Abbvie	Pustular psoriasis	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Biocon/ Mylan	RA; JIA; Plaque psoriasis; PsA; CD; UC	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Boehringer Ingelheim	RA; PsA; Plaque psoriasis; Hidradenitis suppurativa; UC; Pediatric CD	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Coherus	RA; JIA; AS; Plaque psoriasis; PsA; UC	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	EMD Serono/ Merck	Plaque psoriasis	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Fujifilm Kyowa Kirin	RA; JIA; AS; Plaque psoriasis; PsA; UC	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Momenta	RA; Plaque psoriasis	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Oncobiologics/ inVentiv/ Ligand/ Viropro/ Zhejiang Huahai	RA; JIA; AS; PsA; Plaque psoriasis; CD	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Pfizer	RA; JIA; UC; AS; PsA; Plaque psoriasis	SC	Phase 3	TBD
adalimumab (biosimilar to Abbvie's Humira®)	Sandoz	RA; JIA; AS; Plaque psoriasis; PsA; UC	SC	Phase 3	TBD
afatinib (Gilotrif®)	Boehringer Ingelheim	Squamous cell carcinoma; SCCHN; Breast cancer	Oral	Phase 3	TBD
agalsidase beta (biosimilar to Genzyme's Fabrazyme®)	Protalix	Fabry disease	IV	Phase 3	TBD
aglatimagene besadenovec (ProstAtak)	Advantagene	Prostate cancer	Injectable (trans-rectal ultrasound guided)	Phase 3	TBD
algenpantucel-L	NewLink Genetics	Pancreatic cancer	Intradermal	Phase 3; Orphan drug	TBD
alglucosidase alfa	Sanofi	Pompe disease	IV	Phase 3; Orphan drug	TBD
alisporivir (Debio 025)	Debiopharm	Chronic hepatitis C	Oral	Phase 3	TBD
allopregnanolone	SAGE Therapeutics	Super-refractory status epilepticus (SRSE)	IV	Phase 3; Orphan drug	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
amifampridine phosphate (Firdapse)	Biomarin/ Catalyst	Congenital myasthenic syndrome	Oral	Phase 3; Orphan drug	TBD
anifrolumab	AstraZeneca/ Medimmune	Systemic lupus erythematosus	IV	Phase 3	TBD
anlotinib	Jiangsu Chiatai Qingjang	NSCLC; Colorectal cancer; Gastric cancer; Soft tissue sarcoma	Oral	Phase 3	TBD
apalutamide	Janssen	Prostate cancer	Oral	Phase 3	TBD
apatinib	Jiangsu Chiatai Qingijang	Liver cancer	Oral	Phase 3	TBD
arhalofenate	Cymabay Therapeutics	T1DM; T2DM	Oral	Phase 3	TBD
ASP8273	Astellas	NSCLC	Oral	Phase 3	TBD
atacicept	EMD Serono	IgA nephropathy; RA; Systemic lupus erythematosus	SC	Phase 3	TBD
ataluren (Translarna)	PTC Therapeutics	CF in patients with CFTR gene mutations; Becker muscular dystrophy; Duchenne muscular dystrophy	Oral	Phase 3; Orphan drug	TBD
atezolizumab (Tecentriq®)	Genentech/ Roche	Breast cancer; Colorectal cancer; Small-cell lung cancer; Kidney cancer	IV	Phase 3; Orphan drug	TBD
avelumab	Pfizer	NSCLC; Gastric cancer; Ovarian cancer; Urothelial cancer; Diffuse large B-cell lymphoma	IV	Phase 3	TBD
avoralstate	Biocryst	Hereditary angioedema	Oral	Phase 3	TBD
azeliragon	vTv Therapeutics	Alzheimer's disease (Mild to moderate)	Oral	Phase 3	TBD
azeliragonum	Transtech	Alzheimer's disease	Oral	Phase 3	TBD
bavituximab	Peregrine/ AERES Biomedical	NSCLC; Breast cancer	TBD	Phase 3; Fast track (NSCLC)	TBD
belimumab (Benlysta® IV)	GlaxoSmithKline/ Human Genome Sciences	Myasthenia gravis; Refractory idiopathic inflammatory myosistitis; Vasculitis	IV	Phase 3	TBD
bemaciclib	Eli Lilly	Breast cancer	Oral	Phase 3	TBD
benralizumab	Medimmune	Asthma	SC	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
bevacizumab (Avastin®)	Genentech/ Roche	Glioblastoma multiforme	IV	Phase 3	TBD
bevacizumab (biosimilar to Genentech's Avastin®)	AstraZeneca/ Centus Biotherapeutics/ Fujifilm Kyowa Kirin	NSCLC	IV	Phase 3	TBD
bevacizumab (biosimilar to Genentech's Avastin®)	Boehringer Ingelheim	NSCLC; Colorectal cancer; Ovarian cancer; Cervical cancer; Glioblastoma; Kidney cancer	IV	Phase 3	TBD
bevacizumab (biosimilar to Genentech's Avastin®)	Genentech/ Roche	NSCLC; Colorectal cancer; Ovarian cancer; Cervical cancer; Glioblastoma; Kidney cancer	IV	Phase 3	TBD
bevacizumab (biosimilar to Genentech's Avastin®)	Pfizer	NSCLC; Colorectal cancer; Ovarian cancer; Cervical cancer; Glioblastoma; Kidney cancer	IV	Phase 3	TBD
bexagliflozin	Theracos	T1DM; T2DM	Oral	Phase 3	TBD
bilastine	Faes Farma/ Taiho Pharma	Allergic rhinitis; Chronic idiopathic urticaria	Oral	Phase 3	TBD
blisibimod	Anthera	IgA nephropathy; Immune thrombocytopenic purpura; Systemic lupus erythematosus	SC	Phase 3	TBD
bortezomib (Velcade®)	Millennium	ALL	IV	Phase 3; Orphan drug	TBD
brentuximab vedotin (Adcetris®)	Bristol-Myers Squibb/ Millenium/ Seattle Genetics	Cutaneous T-cell lymphoma; Hodgkin's lymphoma; non-Hodgkin's lymphoma; Systemic lupus erythematosus; Diffuse large B-cell lymphoma	IV	Phase 3; Orphan drug	TBD
brodalumab (Siliq)	AstraZeneca/ Valeant	Psoriatic arthritis	SC	Phase 3	TBD
buparlisib	Novartis	Breast cancer	Oral	Phase 3	TBD
busulfan (Busulfex)	Otsuka	Multiple myeloma	Oral	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
cancer peptide vaccine	Agenus/ GlaxoSmithKline	Melanoma	IM	Phase 3	TBD
cannabidiol	Insys	Dravet syndrome; Lennox-Gastaut syndrome	Oral	Phase 3; Orphan drug	TBD
celiprolol	Acer Therapeutics	Ehlers-Danlos syndrome	Oral	Phase 3; Orphan drug	TBD
ceritinib (Zykadia®)	Novartis	NSCLC	Oral	Phase 3; Orphan drug	TBD
certolizumab pegol (Cimzia®)	Dermira/ Royalty/ UCB	JIA; Plaque psoriasis	SC	Phase 3	TBD
cetuximab (Erbitux®)	Bristol-Myers Squibb/ Eli Lilly	NSCLC; SCCHN	IV	Phase 3; Orphan drug	TBD
chiglitazar	Shenzhen Chipscreen Biosciences	T1DM; T2DM	Oral	Phase 3	TBD
cisplatin, liposomally encapsulated (Lipoplatin)	Regulon	NSCLC	Injectable	Phase 3	TBD
copanlisib	Bayer	Non-Hodgkin's lymphoma	Oral	Phase 3	TBD
crenezumab	Genentech/ AC Immune/ Roche	Alzheimer's disease	IV	Phase 3	TBD
crenolanib	Arog	ALL; Gastrointestinal cancer	Oral	Phase 3	TBD
custirsen sodium	Ionis/ OncoGenex	NSCLC; Prostate cancer	IV	Phase 3	TBD
cyclodextrin	Vtesse	Niemann–Pick disease	Intrathecal	Phase 3; Orphan drug	TBD
cysteamine bitartrate (Procysbi®)	Raptor	Huntington's disease; Mitochondrial disorders	Oral	Phase 3	TBD
dabigatran etexilate (Pradaxa®)	Boehringer Ingelheim	Secondary stroke prevention; Stroke prevention in patients with non-valvular atrial fibrillation	Oral	Phase 3	TBD
daclatasvir/ asunaprevir/ beclabuvir	Bristol-Myers Squibb	Hepatitis C	Oral	Phase 3	TBD
dacomitinib	Pfizer	NSCLC	Oral	Phase 3; Orphan drug	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
daratumumab (Darzalex®)	Genmab/ Janssen	Mantel cell lymphoma	IV	Phase 3	TBD
decernotinib	Vertex	RA	Oral	Phase 3	TBD
denosumab (Prolia®)	Amgen/ Daiichi Sankyo	RA	SC	Phase 3	TBD
denosumab (Xgeva®)	Amgen	Multiple myeloma; RA; Pediatric osteogenesis imperfecta (ages 2-17 years)	SC	Phase 3	TBD
deutetrabenazine	Auspex/Teva	Tardive dyskinesia	Oral	Phase 3; Breakthrough therapy	TBD
dimesna disulfide (Tavocept®)	Baxter/ BioNumerik	NSCLC	IV	Phase 3	TBD
dimethyl fumarate	Almirall	Plaque psoriasis	Oral	Phase 3	TBD
dinutuximab beta (biobetter of United Therapeutics' Unituxin™)	Apeiron	Neuroendocrine tumors	IV	Phase 3	TBD
doxorubicin (Livatag)	Bioalliance/ Onxeo	Liver cancer (2nd-line after sorafenib)	IV	Phase 3; Orphan drug	TBD
doxorubicin (ThermoDox)	Celsion/ Hisun	Liver cancer	IV	Phase 3; Orphan drug	TBD
dupilumab	Regeneron/ Sanofi	Asthma; Nasal polyps	SC	Phase 3	TBD
durvalumab	AstraZeneca/ Medimmune	Colorectal cancer; SCCHN	IV	Phase 3; Breakthrough therapy	TBD
duvelisib	Infinity	CLL; Non-Hodgkin's lymphoma	Oral	Phase 3; Orphan drug	TBD
E2609	Biogen/ Eisai	Alzheimer's disease (early)	Oral	Phase 3; Fast track	TBD
eculizumab (Soliris®)	Alexion	Myasthenia gravis; Neuromyelitis optica	IV	Phase 3; Orphan drug	TBD
eflornithine	Orbus	Anaplastic astrocytoma	Oral	Phase 3; Orphan drug	TBD
elafibranor	Genfit	Nonalcoholic steatohepatitis (NASH)	Oral	Phase 3	TBD
eltrapuldencel-T	NeoStem	Melanoma	SC	Phase 3	TBD
encenicline HCl	Forum / Mitsubishi Tanabe	Alzheimer's disease	Oral	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
encorafenib	Array Biopharma	Colorectal cancer; BRAF-mutant melanoma	Oral	Phase 3; Orphan drug	TBD
entinostat	Bayer/ Kyowa Hakko Kirin/ Syndax	Breast cancer	Oral	Phase 3	TBD
enzalutamide (Xtandi®)	Astellas	Prostate (mHNPC)	Oral	Phase 3	TBD
epacadostat	Incyte	Melanoma	Oral	Phase 3; Orphan drug	TBD
epratuzumab	Immunomedics	ALL	IV	Phase 3; Orphan drug	TBD
eribulin mesylate (Halaven®)	Eisai	NSCLC	IV	Phase 3	TBD
ertugliflozin	Merck/ Pfizer	T2DM	Oral	Phase 3	TBD
etanercept (biosimilar to Amgen's Enbrel®)	Coherus/ Daiichi Sankyo	RA; JIA; AS; PsA; Plaque psoriasis	SC	Phase 3	TBD
etirinotecan pegol (Onzeald)	Daiichi-Sankyo/ Nektar	Breast cancer	IV	Phase 3	TBD
etrolizumab	Genentech/ Roche	CD; UC	SC	Phase 3; Orphan drug	TBD
fenfluramine	Zogenix	Dravet syndrome	Oral	Phase 3; Orphan drug	TBD
fevipiprant	Novartis	Eosinophilic asthma	Oral	Phase 3	TBD
filgotinib	Galapagos/ Gilead	CD; UC	Oral	Phase 3	TBD
fingolimod (Gilenya®)	Mitsubishi Tanabe/ Novartis	Primary progressive MS	Oral	Phase 3	TBD
fosbretabulin tromethamine	Oxigene	Ovarian cancer	IV	Phase 3	TBD
fostamatinib	Rigel	Immune thrombocytopenic purpura	Oral	Phase 3; Orphan drug	TBD
fruquintinib	Eli Lilly/ Hutchison Medipharma	Colorectal cancer	Oral	Phase 3	TBD
galeterone	Tokai	Prostate cancer	Oral	Phase 3	TBD
ganetespib	Synta	ALL; NSCLC	IV	Phase 3	TBD
gantenerumab	Genentech/ Morphosys/ Roche	Alzheimer's disease	SC	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
gemigliptin	LG Life Sciences/ Sanofi	T1DM; T2DM	Oral	Phase 3	TBD
gilteritinib	Astellas/ Kotobuki	AML	Oral	Phase 3	TBD
givinostat	Italfarmaco	Duchenne muscular dystrophy	Oral	Phase 3; Orphan drug	TBD
glufosfamide	Eleison	Pancreatic cancer	IV	Phase 3; Orphan drug	TBD
golimumab (Simponi®)	Janssen	JIA	SC	Phase 3; Orphan drug	TBD
grazoprevir / elbasvir (Zepatier®)	Merck	Hepatitis C gentoype 6	Oral	Phase 3	TBD
guadecitabine	Astex/ Otsuka	Chronic myelomonocytic leukemia (CMML); Myelodysplastic syndromes; AML	SC	Phase 3	TBD
hypericin	Soligenix	Cutaneous T-cell lymphoma	Topical	Phase 3; Orphan drug	TBD
ibrutinib (Imbruvica®)	Janssen/ Pharmacyclics	Diffuse large B cell lymphoma; Pancreatic cancer; B-cell lymphoma; Graft versus host disease	Oral	Phase 3; Orphan drug	TBD
icotinib HCl	Beta Pharma	NSCLC	Oral	Phase 3	TBD
idalopirdine	H. Lundbeck/ Otsuka	Alzheimer's disease (mild to moderate)	Oral	Phase 3	TBD
idebenone (Raxone)	Santhera	Duchenne muscular dystrophy; Leber's hereditary optic neuropathy	Oral	Phase 3; Orphan drug	TBD
idursulfase-IT	Shire	Hunter syndrome	Intrathecal	Phase 3; Orphan drug	TBD
imetelstat	Janssen	Myelodysplastic syndromes	IV	Phase 3; Orphan drug	TBD
immune globulin (Hyqvia®)	Baxalta/ Halozyme Therapeutics/ Shire	Chronic inflammatory demyelinating polyneuritis (CIDP)	IV	Phase 3	TBD
inebilizumab	AstraZeneca	Neuromyelitis optica	IV	Phase 3	TBD
infliximab (biosimilar to Janssen's Remicade®)	Amgen	RA	IV	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
infliximab (biosimilar to Janssen's Remicade®)	Pfizer	RA; AS; CD; UC; PsA; Plaque psoriasis	SC	Phase 3	TBD
inotuzumab ozogamicin	Pfizer	ALL	IV	Phase 3; Orphan drug	TBD
intepirdine	Axovant/ Roivant	Alzheimer's disease (mild to moderate)	Oral	Phase 3	TBD
interferon gamma-1b (Actimmune®)	Horizon/ InterMune	Friedreich's ataxia	SC	Phase 3; Orphan drug	TBD
isatuximab	Immunogen/ Sanofi	Multiple myeloma	IV	Phase 3	TBD
ixazomib citrate (Ninlaro®)	Millennium/ Takeda	ALL; Multiple myeloma; Non-Hodgkin's lymphoma; Systemic light chain amyloidosis	Oral	Phase 3; Orphan drug	TBD
ixekizumab (Taltz®)	Eli Lilly	Axial spondyloarthritis; Genital psoriasis; PsA	SC	Phase 3	TBD
lanreotide acetate (Somatuline Depot®)	Ipsen	Carcinoid syndrome	SC	Phase 3; Orphan drug	TBD
laquinimod (Nerventra)	Active Biotech/ Teva	Relapsing-remitting MS	Oral	Phase 3	TBD
lasmiditan	Colucid	Migraine	Oral, IV	Phase 3	TBD
lebrikizumab	Genentech/ Roche	Asthma	SC	Phase 3	TBD
lercanidipine/ valsartan (ZV Combi)	LG Life Sciences	Hypertension	Oral	Phase 3	TBD
lurbinectedin	Pharmamar	Ovarian cancer; Small cell lung cancer	IV	Phase 3; Orphan drug	TBD
luseogliflozin hydrate	Novartis/ Taisho	T1DM; T2DM	Oral	Phase 3	TBD
margetuximab	Macrogenics	Breast cancer (HER2-positive)	IV	Phase 3	TBD
masitinib	AB Science	Asthma; Alzheimer's disease; Amyotrophic lateral sclerosis; Colorectal cancer; Gastric cancer; Melanoma; Primary progressive MS; Relapse-free secondary progressive MS; RA	Oral	Phase 3; Orphan drug	TBD
melphalan (Melblez Kit)	Delcath	Ocular melanoma metastatic to the liver	Infusion (isolated)	Phase 3; Orphan drug	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
mepolizumab (Nucala®)	GlaxoSmithKline	Churg–Strauss syndrome; Hypereosinophilic syndrome	SC	Phase 3; Orphan drug	TBD
methylthioninium	Taurx	Alzheimer's disease	Oral	Phase 3	TBD
migalastat (Galafold)	Amicus	Fabry disease	Oral	Phase 3; Orphan drug	TBD
mitomycin (MitoGel)	Urogen	Urothelial cancer	Intravesical	Phase 3; Orphan drug	TBD
momelotinib	Gilead	Myelofibrosis; Pancreatic cancer	Oral	Phase 3	TBD
mongersen	Celgene/ Nogra	CD	Oral	Phase 3; Orphan drug	TBD
napabucasin	Boston Biomedical/ Sumitomo Dainippon	NSCLC; Colorectal cancer; Gastric cancer; Gastroesophageal junction cancer	Oral	Phase 3; Orphan drug	TBD
natalizumab (Tysabri®)	Biogen/ Elan	Secondary progressive MS	IV	Phase 3	TBD
neratinib	Puma Biotechnology	NSCLC; Solid tumors	Oral	Phase 3	TBD
nimotuzumab	Daiichi Sankyo/ Innomab	NSCLC	IV	Phase 3	TBD
nintedanib (Ofev®)	Boehringer Ingelheim	NSCLC; Systemic sclerosis with lung involvement; Ovarian cancer; Colorectal cancer	Oral	Phase 3; Orphan drug	TBD
niraparib	Janssen/ Tesaro	Breast cancer	Oral	Phase 3	TBD
nivolumab (Opdivo®)	Bristol-Myers Squibb/ Ono	NSCLC; Urothelial cancer; Gastroesophageal cancer; Gastric cancer; Glioblastoma (recurrent)	IV	Phase 3	TBD
NKTR-214	Nektar	Breast cancer	Injectable	Phase 3	TBD
obeticholic acid (Ocaliva®)	Intercept	Nonalcoholic steatohepatitis (NASH)	Oral	Phase 3	TBD
obinutuzumab (Gazyva®)	Genentech/ Roche	Diffuse large B-cell lymphoma; Front-line CLL	IV	Phase 3; Orphan drug	TBD
ODM-201	Bayer/ Orion	Prostate cancer	Oral	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
ofatumumab SC (Arzerra® SC)	Novartis/ Stiefel Labs	MS	SC	Phase 3	TBD
olaparib (Lynparza®)	AstraZeneca	Breast cancer; Pancreatic cancer	Oral	Phase 3	TBD
omarigliptin (Marizev)	Merck	T1DM; T2DM	Oral	Phase 3	TBD
ombitasvir/ paritaprevir/ ritonavir (Technivie™)	Abbvie	Hepatitis C genotype 1; Hepatitis C genotype 1 and 4 (pediatric)	Oral	Phase 3; Orphan drug	TBD
opicapone (Ongentys)	Bial	Parkinson's disease	Oral	Phase 3	TBD
ozanimod	Receptos/ Celgene	Relapsing MS; UC	Oral	Phase 3; Orphan drug	TBD
paclitaxel	Nippon Kayaku	Breast cancer	IV	Phase 3	TBD
paclitaxel	Sun	Biliary cancer	IV	Phase 3	TBD
paclitaxel (Cynviloq)	Nantpharma/ Nantworks/ Sorrento	Breast cancer	IV	Phase 3	TBD
pacritinib	Baxalta/ CTI Biopharma/ Shire	Myelofibrosis	Oral	Phase 3; Orphan drug	TBD
panobinostat lactate (Farydak®)	Novartis	Hodgkin's lymphoma; Cutaneous T-cell lymphoma	Oral	Phase 3	TBD
patisiran	Alnylam / Arbutus/ Sanofi	Familial amyloidotic polyneuropathy	IV	Phase 3	TBD
peficitinib hydrobromide	Astellas/ Janssen	RA	Oral	Phase 3	TBD
peginterferon alfa-2b (Algeron)	Biocad	Hepatitis C infection	SC	Phase 3	TBD
peginterferon alfa-2b (biobetter to Merck's Peg-Intron®)	Pharmaessentia	Hepatitis C infection	SC	Phase 3	TBD
peginterferon lambda-1a	Bristol-Myers Squibb	Hepatitis C infection	SC	Phase 3	TBD
pegpleranib sodium (Fovista)	Genentech/ Novartis/ Ophthotech	Wet age-related macular degeneration	Intravitreal	Phase 3	TBD
pelareorep (Reolysin)	Oncolytics	SCCHN	IV	Phase 3	TBD
pembrolizumab (Keytruda®)	Merck	Breast cancer; Bladder cancer; Colorectal cancer; Gastric cancer; Esophageal cancer; Head/neck cancer; Multiple myeloma	IV	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
personalized anti-idiotype vaccine (BiovaxID)	Biovest International	Follicular lymphoma; Mantel cell lymphoma	SC	Phase 3	TBD
piclidenoson	Can-Fite Biopharma	RA; Plaque psoriasis	Oral	Phase 3	TBD
pixantrone dimaleate (Pixuvri)	CTI Biopharma	Diffuse large B cell lymphoma; Follicular lymphoma	IV	Phase 3	TBD
plinabulin	Beyondspring	NSCLC	IV	Phase 3	TBD
plitidepsin (Aplidin)	Pharmamar	Multiple myeloma	IV	Phase 3; Orphan drug	TBD
ponesimod	Actelion	Relapsing MS	Oral	Phase 3	TBD
pradigastat	Novartis	Familial chylomicronemia syndrome; Hyperlipoproteinemia; Hypertriglyceridemia; T1DM; T2DM; CAD	Oral	Phase 3	TBD
quizartinib	Ambit Biosciences/ Daiichi Sankyo	AML	Oral	Phase 3	TBD
ramucirumab (Cyramza®)	Eli Lilly	Hepatocellular carcinoma; Bladder cancer	IV	Phase 3; Orphan drug	TBD
ranibizumab (biosimilar to Genentech's Lucentis®)	Formycon/ bioeq/ Polpharma/ Santo Holding/ Swiss Pharma	Wet age-related macular degeneration; Macular edema; Diabetic macular edema; Diabetic retinopathy	Intravitreal	Phase 3	TBD
remestemcel-L (Prochymal)	Mesoblast	CD; Graft versus host disease	IV	Phase 3	TBD
reslizumab (Cinqair®)	Teva	Chronic rhinosinusitis	IV	Phase 3	TBD
reslizumab SC	Teva	Eosinophilic asthma	SC	Phase 3	TBD
retagliptin	Jiangsu Hengrui Medical	T1DM; T2DM	Oral	Phase 3	TBD
revefenacin	Mylan/ Theravance	COPD	Inhalation	Phase 3	TBD
reveglucosidase alfa	Biomarin	Pompe disease (Late [non-infantile] onset)	IV	Phase 3	TBD
rigerimod (Lupuzor)	Immupharma	Systemic lupus erythematosus	SC	Phase 3; Fast track	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
rigosertib	Onconova	Myelodysplastic syndromes; Pancreatic cancer	IV	Phase 3; Orphan drug	TBD
rilimogene galvacirepvec/ rilimogene glafolivec (Prostvac)	Bristol-Myers Squibb/ Bavarian Nordic	Prostate cancer	SC	Phase 3	TBD
risankizumab	Abbvie/ Boehringer Ingelheim	Psoriasis	SC	Phase 3	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Archigen/ AstraZeneca/ Samsung	Follicular lymphoma	IV	Phase 3; Orphan drug	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Allergan/ Amgen	CLL; Granulomatosis with polyangiitis; RA; Non-Hodgkin's lymphoma	IV	Phase 3; Orphan drug	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Celltrion/ Teva	CLL; RA; Granulomatosis with polyangiitis; Non-Hodgkin's lymphoma	IV	Phase 3; Orphan drug	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Pfizer	CLL; Granulomatosis with polyangiitis; Follicular lymphoma; Non-Hodgkin's lymphoma; RA	IV	Phase 3	TBD
rituximab (biosimilar to Genentech's Rituxan®)	Sandoz	Follicular lymphoma; RA	IV	Phase 3; Orphan drug	TBD
rose bengal disodium	Provectus	Melanoma	Intralesional	Phase 3; Orphan drug	TBD
rucaparib	Clovis Oncology	Fallopian tube cancer; Peritoneal cancer	Oral	Phase 3	TBD
sacituzumab govitecan	Immunomedics	Breast cancer	IV	Phase 3	TBD
sarizotan	Newron	Rett syndrome	Oral	Phase 3; Orphan drug	TBD
secukinumab (Cosentyx®)	Novartis	Axial spondyloarthritis; RA	SC	Phase 3	TBD
selumetinib sulphate	Array Biopharma/ AstraZeneca	NSCLC; Melanoma; Differentiated thyroid cancer; Neurofibromatosis type 1	Oral	Phase 3; Orphan drug	TBD
seviprotimut-L	CK Life Sciences	Melanoma	Intradermal	Phase 3	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
siponimod	Novartis	Secondary progressive MS	Oral	Phase 3	TBD
sirukumab	Eli Lilly/ GlaxoSmithKline/ Janssen	Temporal arteritis; Polymyalgia rheumatica	SC	Phase 3	TBD
sorafenib tosylate (Nexavar®)	Bayer	ALL; Breast cancer; Melanoma	Oral	Phase 3	TBD
sotagliflozin	Lexicon/ Sanofi	T1DM; T2DM	Oral	Phase 3	TBD
tafamidis meglumine	Pfizer	Transthyretin (TTR) mediated amyloidosis	Oral	Phase 3; Orphan drug	TBD
talazoparib	Medivation	Breast cancer	Oral	Phase 3	TBD
taselisib	Chugai/ Genentech/ Roche	Breast cancer	Oral	Phase 3	TBD
tasquinimod	Active Biotech/ Ipsen	Prostate cancer	Oral	Phase 3	TBD
tegafur/ gimeracil/ oteracil	Otsuka/ Taiho Pharma/ Yakult Honsha	Gastric cancer	Oral	Phase 3; Orphan drug	TBD
teneligliptin (Tenelia)	Mitsubishi Tanabe	T1DM; T2DM	Oral	Phase 3	TBD
teneligliptin/ canagliflozin	Mitsubishi Tanabe	T1DM; T2DM	Oral	Phase 3	TBD
teplizumab	MacroGenics	T1DM; T2DM	IV	Phase 3	TBD
tesevatinib	Kadmon	NSCLC	Oral	Phase 3	TBD
tezacaftor	Vertex	CF in patients with CFTR gene mutations	Oral	Phase 3	TBD
Tg-4010	Transgene/ Virax	NSCLC	Injectable	Phase 3	TBD
TGF-Beta Antisense	3M Innovative	NSCLC	Injectable	Phase 3	TBD
TGR-1202	TG Therapeutics	CLL; Diffuse large B cell lymphoma	Oral	Phase 3	TBD
tildrakizumab	Merck/ Sun	Plaque psoriasis	SC	Phase 3	TBD
tirasemtiv	Cytokinetics	Amyotrophic lateral sclerosis	Oral	Phase 3; Orphan drug	TBD
tivantinib	Arqule/ Kyowa/ Daiichi Sankyo	NSCLC; Liver cancer	Oral	Phase 3; Orphan drug	TBD
tivozanib	Aveo/ Kyowa/ Ophthotech	Kidney cancer	Oral	Phase 3	TBD
tocilizumab (Actemra®)	Genentech/ Roche	RA; Systemic sclerosis; Osteroarthritis of the hand	SC	Phase 3; Orphan drug	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
tofacitinib (Xeljanz®)	Pfizer/ Takeda	Psoriatic arthritis; JIA; UC	Oral	Phase 3	TBD
tofogliflozin	Chugai/ Genentech/ Kowa/ Roche/ Sanofi	T1DM; T2DM	Oral	Phase 3	TBD
tozadenant	Biotie Therapies	Parkinson's disease	Oral	Phase 3	TBD
trabectedin (Yondelis®)	Janssen/ Pharmamar	Ovarian cancer; Peritoneal cancer; Fallopian tube cancer; Soft tissue sarcoma	IV	Phase 3; Orphan drug	TBD
tralokinumab	AstraZeneca/ Leo/ Medimmune	Asthma	SC	Phase 3	TBD
tramiprosate	Alzheon	Alzheimer's disease	Oral	Phase 3	TBD
trastuzumab (biosimilar to Genentech's Herceptin®)	Celltrion/ Teva	Breast cancer (HER2-positive); Gastric cancer; Gastroesophageal junction cancer	IV	Phase 3	TBD
trastuzumab (biosimilar to Genentech's Herceptin®)	Pfizer	Breast cancer (HER2-positive); Gastric cancer; Gastroesophageal cancer	IV	Phase 3; Orphan drug	TBD
trastuzumab SC (Herceptin® SC)	Genentech/ Roche	Breast cancer (HER2-positive)	SC	Phase 3	TBD
trebananib	Amgen	Ovarian cancer	IV	Phase 3; Orphan drug	TBD
trehalose	Bioblast	Oculopharyngeal muscular dystrophy	IV	Phase 3; Orphan drug	TBD
trelagliptin succinate	Takeda	T1DM; T2DM	Oral	Phase 3	TBD
tremelimumab	AstraZeneca/ Medimmune	NSCLC; SCCHN; Bladder cancer	IV	Phase 3	TBD
trifluridine/ tipiracil HCl (Lonsurf®)	Otsuka/ Taiho	Gastric cancer	Oral	Phase 3	TBD
turgenpumatucel-L	Newlink Genetics	NSCLC	Injectable	Phase 3	TBD
ublituximab	TG Therapeutics	CLL	IV	Phase 3; Orphan drug	TBD
udenafil	Allergan/ Mezzion	Alzheimers disease pyschosis; Erectile dysfunction; Fontan palliation; Pulmonary arterial hypertension	Oral	Phase 3; Orphan drug	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
upadacinitib	Abbvie	RA	Oral	Phase 3	TBD
ustekinumab SC (Stelara® SC)	Janssen	AS; UC	IV	Phase 3	TBD
veliparib	Abbvie	NSCLC; Brain cancer; Breast cancer; Ovarian cancer	Oral	Phase 3; Orphan drug	TBD
venetoclax (Venclexta®)	Abbvie/ Genentech/ Roche	Multiple myeloma	Oral	Phase 3; Orphan drug	TBD
vercirnon	Chemocentryx	CD	Oral	Phase 3	TBD
verubecestat	Merck	Alzheimer's disease	Oral	Phase 3	TBD
vildagliptin	Novartis	T1DM; T2DM	Oral	Phase 3	TBD
vocimagene amiretrorepvec/ flucytosine	Tocagen	Anaplastic astrocytoma; Glioblastoma multiforme	Oral, IV	Phase 3	TBD
volanesorsen	Akcea/ Ionis	Familial chylomicronemia syndrome; Hypertriglyceridemia; Partial lipodystrophy	SC	Phase 3; Orphan drug	TBD
volasertib	Boehringer Ingelheim	AML	IV	Phase 3; Orphan drug	TBD
vosaroxin (Qinprezo)	Sunesis	ALL	IV	Phase 3; Orphan drug	TBD
zoledronic acid (Orazol)	Merrion	Breast cancer	Oral	Phase 3	TBD
zoptarelin doxorubicin	Aeterna Zentaris	Endometrial cancer	IV	Phase 3	TBD
Complete Response Letter (CRL) / Withdrawn Drugs					
amifampridine phosphate (Firdapse)	Biomarin/ Catalyst	Lambert-Eaton myasthenic syndrome	Oral	CRL; Breakthrough therapy; Orphan Drug	TBD
andexanet alfa (Andexxa)	Portola	Factor Xa inhibitor reversal	IV	CRL; Breakthrough therapy; Orphan Drug; Priority review	TBD
apaziquone (Qapzola)	Spectrum	Bladder cancer	Intravesical	CRL	TBD
aripiprazole (Abilify sensor tablet)	Otsuka/ Proteus Digital Health	Bipolar I (acute); Major depressive disorder; Schizophrenia	Oral	CRL	TBD

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
benzhydrocodone/ acetaminophen (Apadaz)	Kempharm	Acute pain (short-term)	Oral	CRL	TBD
bivalirudin (Kangio)	Eagle	Blood clot prevention in patients undergoing PCI; Unstable angina in patients undergoing coronary angioplasty	IV	CRL	TBD
cetirizine	Nicox	Allergic conjunctivitis	Ophthalmic solution	CRL	TBD
dexamethasone SR 0.4% ocular insert (Dextenza)	Ocular Therapeutix/ Ora	Post-surgical ocular inflammation	Ophthalmic implant	CRL	TBD
hepatitis B vaccine (Heplisav-B)	Dynavax	Hepatitis B immunization	IM	CRL	N/A
immune globulin	ADMA Biologics	Primary immunodeficiency	IV	CRL	TBD
immune globulin	Green Cross	Primary immunodeficiency	IV	CRL	TBD
insulin aspart (ultrafast acting)	Novo Nordisk	T1DM; T2DM	SC	CRL	TBD
latanoprostene bunod (Vesneo)	Bausch & Lomb/ Nicox	Glaucoma; Ocular hypertension	Ophthalmic	CRL	TBD
lutetium Lu 177 dotatate (Lutathera)	Advanced Accelerator Applications/ Fujifilm	Gastro-entero pancreatic neuroendocrine tumors (NET)	IV	CRL; Fast track; Orphan drug; Priority review	TBD
MCNA	Telesta	Bladder cancer	Intravesical	CRL	TBD
octreotide (Mycapssa)	Chiasma	Acromegaly	Oral	CRL; Orphan Drug	N/A
odanacatib	Merck/ Quest Diagnostics	Osteoporosis	Oral	Withdrawn	N/A
oxycodone/ naltrexone abuse-deterrent (SequestOx)	Elite	Moderate to severe pain	Oral	CRL; Priority review	TBD
oxydocone long-acting abuse-deterrent (Remoxy)	Durect Pain Therapeutics	Chronic pain	Oral	CRL	TBD
pegfilgrastim	Sandoz	Neutropenia in patients receiving myelosuppressive chemotherapy	SC	CRL	TBD
revusiran	Sanofi	Familial amyloidotic cardiomyopathy	SC	Withdrawn	N/A

PIPELINE DRUG LIST cont.

NAME	MANUFACTURER	CLINICAL USE	DOSAGE FORM	APPROVAL STATUS	EXPECTED FDA APPROVAL
rociletinib hydrobromide	Clovis Oncology	NSCLC	Oral	Withdrawn; Orphan drug	N/A
sarilumab	Regeneron/ Sanofi	RA	SC	CRL	TBD
solanezumab	Eli Lilly	Alzheimer's disease	IV	Withdrawn	N/A
solithromycin	Cempra	Community-acquired bacterial pneumonia (CABP)	Oral, IV	CRL	TBD
testosterone undecanoate	Lipocine	Testosterone deficiency	Oral	CRL	TBD
velcalcetide (Parsabiv)	Amgen	Secondary hyperparathyroidism associated with chronic kidney disease	IV	CRL	TBD

GLOSSARY

ADHD Attention Deficit Hyperactivity Disorder

ALL Acute Lymphoblastic Leukemia

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

CD Crohn's Disease

CF Cystic Fibrosis

CKD Chronic Kidney Disease

COPD Chronic Obstructive Pulmonary Disease

CRL Complete Response Letter

CV Cardiovascular

CVD Cardiovascular Disease

FDA Food and Drug Administration

ER Extended-release

GLP-1 Glucagon-like Peptide-1

H Half

HCP Healthcare Professional

HIT Heparin Induced Thrombocytopenia

HTN Hypertension

IM Intramuscular

IV Intravenous

JIA Juvenile Idiopathic Arthritis

LDL-C Low-density Lipoprotein Cholesterol

MS Multiple Sclerosis

N/A Not Applicable

NDA New Drug Application

NSCLC Non-Small Cell Lung Cancer

PCI Percutaneous Coronary Intervention

PDUFA Prescription Drug User Fee Act

PSA Psoriatic Arthritis

PTCA Percutaneous Transluminal Coronary Angioplasty

Q Quarter

RA Rheumatoid Arthritis

sBLA Supplemental Biologics License Application

SC Subcutaneous

SCCHN Squamous Cell Cancer of the Head and Neck

sNDA Supplemental New Drug Application

T1DM Type 1 Diabetes Mellitus

T2DM Type 2 Diabetes Mellitus

TBD To Be Determined

UA Unstable Angina

UC Ulcerative Colitis

US United States

XR Extended-release