OPTUMRx®

RxOutlook®

4th Quarter 2020

While COVID-19 vaccines draw most attention, multiple "firsts" are expected from the pipeline in 1Q:2021

Great attention is being given to pipeline drugs that are being rapidly developed for the treatment or prevention of SARS-CoV-19 (COVID-19) infection, particularly two vaccines that are likely to receive emergency use authorization (EUA) from the Food and Drug Administration (FDA) in the near future. Earlier this year, FDA issued a Guidance for Industry that indicated the FDA expected any vaccine for COVID-19 to have at least 50% efficacy in preventing COVID-19. In November, two manufacturers, Pfizer and Moderna, released top-line results from interim analyses of their investigational COVID-19 vaccines. Pfizer stated their vaccine, BNT162b2 had demonstrated > 90% efficacy. Several days later, Moderna stated their vaccine, mRNA-1273, had demonstrated 94% efficacy. Many unknowns still exist, such as the durability of response, vaccine performance in vulnerable sub-populations, safety, and tolerability in the short and long term. Considering the first U.S. case of COVID-19 was detected less than 12 months ago, the fact that two vaccines have far exceeded the FDA's guidance and are poised to earn EUA clearance, is remarkable. If the final data indicates a positive risk vs. benefit profile and supports final FDA clearance, there may be lessons from this accelerated development timeline that could be applied to the larger drug development pipeline in the future.

Meanwhile, drug development in other areas continues. In this edition of RxOutlook, we highlight 12 key pipeline drugs with potential to launch by the end of the first quarter of 2021. Of note, there are multiple "firsts" described in this report. Vericiguat is the first in a new class of cardiovascular drugs called soluble guanylate cyclase stimulators and could be approved for use in combination with existing heart failure regimens. Voclosporin could be the first drug approved by the FDA for lupus nephritis, an area that could see more attention later in 2021 when Benlysta® (belimumab) receives an approval decision for a new indication for lupus nephritis. Aducanumab could be the first disease-modifying treatment for Alzheimer's disease, a common condition with high disease burden, limited treatment options, and high unmet need. However, the efficacy data supporting aducanumab is not as strong as some clinicians had hoped, and its prospects for approval unclear, but given the potential impact this drug could have, it certainly warrants watching. Finally, idecabtagene vicleucel could be the first CAR T cell therapy to receive FDA approval for multiple myeloma. So far, CAR T cell therapies have been limited to lymphomas, but that could change in 2021 as two multiple myeloma CAR T cell therapies are expected to hit the U.S. market, idecabtagene vicleucel in 1Q 2021 and later ciltacabtagene autoleucel in mid-2021.

Key pipeline drugs with FDA approval decisions expected by the end of the 1st quarter 2021

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Dostarlimab	GlaxoSmithKline	Endometrial cancer	4Q 2020
Vericiguat	Merck/Bayer	Heart failure	1/20/2021
Voclosporin	Aurinia Pharmaceuticals	Lupus nephritis	1/22/2021
Pegunigalsidase alfa	Protalix	Fabry disease*	1/27/2021
Evinacumab	Regeneron	Homozygous familial hypercholesterolemia*	2/11/2021
Umbralisib	TG Therapeutics	Marginal zone lymphoma (MCL)* / Follicular lymphoma (FL)*	2/15/2021 (MCL) / 6/15/2021 (FL)
Casimersen	Sarepta	Duchenne muscular dystrophy*	2/25/2021
Aducanumab	Biogen	Alzheimer's disease	3/7/2021

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Arimoclomol	Orphazyme	Niemann-Pick disease type C*	3/17/2021
Ponesimod	Janssen	Multiple sclerosis	3/18/2021
Idecabtagene vicleucel	Bristol-Myers Squibb/ bluebird bio	Multiple myeloma*	3/27/2021
Belumosudil	Kadmon	Graft vs. host disease*	1Q 2021

* Orphan Drug Designation

OptumRx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook. The report is organized in the following manner:

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 4th quarter 2020.

Read more

Extended Generic Pipeline Forecast

This section provides a summary of upcoming first-time generic drugs and biosimilars that may be approved in the upcoming two years.

Read more

Extended Brand Pipeline Forecast

This supplemental table provides a summary of developmental drugs, including both traditional and specialty medications that may be approved in the upcoming two years.

Read more

Key Pending Indication Forecast

This supplemental table provides a summary of key new indications that are currently under review by the FDA and may be approved in the upcoming 12 months.

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 1st quarter 2021 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Getting acquainted with pipeline forecast terms

Clinical trial phases

Phase I trials	Researchers test an experimental drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.
Phase II trials	The experimental study drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.
Phase III trials	The experimental study drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
Phase IV trials	Post marketing studies delineate additional information including the drug's risks, benefits, and optimal use.

Pipeline acronyms

ANDA	Abbreviated New Drug Application
BLA	Biologic License Application
CRL	Complete Response Letter
FDA	Food and Drug Administration
MOA	Mechanism of Action
NME	New Molecular Entity
NDA	New Drug Application
sBLA	Supplemental Biologic License Application
sNDA	Supplemental New Drug Application
OTC Drugs	Over-the-Counter Drugs
PDUFA	Prescription Drug User Fee Act
REMS	Risk Evaluation and Mitigation Strategy

Detailed insights on key drugs

Dostarlimab (Brand Name: To be determined)

Manufacturer: GlaxoSmithKline
FDA approval date: 4Q 2020

Therapeutic use

Dostarlimab is in development for the treatment of women with mismatch repair-deficient (dMMR)/microsatellite instability high (MSI-H) recurrent or advanced endometrial cancer.

Cancer of the endometrium is the most common cancer of the female reproductive organs. There are approximately 60,000 new cases of endometrial cancer diagnosed annually and it affects mainly post-menopausal women. The 5-year relative survival rates for regional and distant endometrial cancer are 69% and 17%, respectively.

An estimated 25% of patients with endometrial cancer have the dMMR/MSI-H biomarker. In normal cells, a system called DNA MMR corrects errors that occur during DNA replication. Defects in MMR can lead to MSI-H, which can be found in many types of cancer. These tumors have accumulation of errors in genetic sequences that are normally repeated (called microsatellites).

- Treatment of women with dMMR/MSI-H recurrent or advanced endometrial cancer

Clinical profile

Dostarlimab is an anti-programmed death (PD)-1 monoclonal antibody that binds with high affinity to the PD-1 receptor and effectively blocks its interaction with the ligands PD-L1 and PD-L2. PD-1 and PD-L1/2 interaction directly inhibits apoptosis of tumor cells.

Pivotal trial data:
The efficacy of dostarlimab was evaluated in the GARNET trial, a Phase 1/2, single-arm study that included a subset of patients with endometrial cancer and the dMMR/MSI-H biomarker. A total of 71 patients had ≥ 6 months of follow-up by the data cutoff. The primary endpoints were objective response rate (ORR) and duration of response (DOR) as assessed using RECIST v1.1 criteria. Treatment with dostarlimab showed an ORR of 42% (95% CI: 31, 55). At the time of data cutoff, with a median follow up of 11.2 months, the median DOR had not been reached (1.87+ to 19.61+ months).

Safety:
The most common adverse events with dostarlimab use were asthenia, diarrhea, fatigue, and nausea.

Dosing:
In the pivotal trial, dostarlimab was administered via intravenous (IV) infusion every 3 weeks for 4 cycles, then every 6 weeks thereafter.

- Anti-PD-1 monoclonal antibody
- IV formulation
- ORR: 42%
- Common AEs: Asthenia, diarrhea, fatigue, nausea
- Dosing: Every 3 weeks for 4 cycles then every 6 weeks thereafter

Dostarlimab (continued...)

Competitive environment

If approved, dostarlimab would provide an additional single-agent treatment option for endometrial cancer and the second therapy for patients with this specific biomarker. The only other drug approved for patients with endometrial cancer with dMMR/MSI-H is Keytruda® (pembrolizumab). Dostarlimab is also being evaluated for additional uses, including patients with solid tumors and dMMR/MSI-H.

The initial indication for dostarlimab would be narrow and it would be competing with Keytruda which has been on the market since 2014 and approved for 19 different indications and uses. Additionally, the information currently available for dostarlimab are from an early stage trial and there is a lack of robust overall survival (OS) and progression-free survival (PFS) data.

For reference, the Wholesale Acquisition Cost (WAC) for Keytruda is $19,739 per dose every 6 weeks.

- Advantages: Additional single-agent treatment option for endometrial cancer, potential future uses (eg, other dMMR/MSI-H solid tumors)
- Disadvantages: Alternative available, narrow initial indication, lack of OS/PFS data
- Reference WAC (Keytruda): $19,739 per dose every 6 weeks

Vericiguat (Brand Name: To be determined)

Manufacturer: Merck/Bayer
FDA approval date: 1/20/2021

Therapeutic use

Vericiguat is in development for the reduction of risk of cardiovascular death (CVD) and heart failure hospitalization following a worsening heart failure event in patients with symptomatic chronic heart failure with reduced ejection fraction (HFrEF), in combination with other heart failure therapies.

HFrEF, or systolic heart failure, is a category of heart failure in which the heart is unable to eject blood sufficiently during its contraction phase. Of the estimated 6.2 million people in the U.S. with heart failure, approximately 50% have HFrEF.

Despite available treatment options, approximately 50% of patients hospitalized for heart failure are readmitted within 6 months, and almost 30% die within a year.

- Reduce the risk of CVD and heart failure hospitalization following a worsening heart failure event in patients with symptomatic HFrEF, in combination with other heart failure therapies

Clinical profile

Vericiguat is a soluble guanylate cyclase (sGC) stimulator. It works by enhancing the cyclic guanosine monophosphate (cGMP) pathway by directly stimulating sGC through a binding site independent of nitric oxide, and it sensitizes sGC to endogenous nitric oxide by stabilizing nitric oxide binding to the binding site.

Experimental studies have suggested multiple potential benefits of sGC stimulators including prevention of left ventricular hypertrophy and fibrosis, as well as reduction of ventricular afterload through vasodilation.

- sGC stimulator
- Oral formulation
- Composite of CVD or first hospitalization for heart failure: 35.5% vs. 38.5% of patients in the placebo arm
- Common AEs: Hypotension, syncope
- Dosing: Once daily

Vericiguat (continued...)

Pivotal trial data:

The efficacy of vericiguat was evaluated in the VICTORIA trial, a Phase 3, randomized, placebo-controlled, double-blind study in 5,050 patients with chronic heart failure (New York Heart Association class II, III, or IV) and an ejection fraction of less than 45%. Patients received vericiguat or placebo, in addition to guideline-based medical therapy. About 15% of patients were receiving background therapy with Entresto® (sacubitril/valsartan). The primary outcome was a composite of death from cardiovascular causes or first hospitalization for heart failure.

Over a median of 10.8 months, a primary-outcome event occurred in 35.5% of patients in the vericiguat group vs. 38.5% of patients in the placebo group (hazard ratio [HR] 0.90, 95% CI: 0.82, 0.98; p = 0.02). In the vericiguat group, 27.4% were hospitalized for heart failure vs. 29.6% in the placebo group (HR 0.90, 95% CI: 0.81, 1.00). CVD occurred in 16.4% of patients the vericiguat group and in 17.5% of patients in the placebo group (HR 0.93, 95% CI: 0.81, 1.06; not statistically significant). Death from any cause occurred in 20.3% and 21.2% of patients receiving vericiguat and placebo, respectively (HR 0.95, 95% CI: 0.84, 1.07; p = 0.38; not statistically significant).

Safety:

The most common adverse events with vericiguat use were symptomatic hypotension and syncope.

Dosing:

In the pivotal trial, vericiguat was administered orally once daily.

Competitive environment

Vericiguat is a first-in-class, oral sGC stimulator and if approved, would offer an additional treatment option in the treatment of chronic heart failure. Despite the availability of existing treatment options, there is still a high unmet need for better treatments for heart failure. Based on data from the CDC, heart failure was mentioned on 379,800 death certificates in 2018 and heart failure costs an estimated $30.7 billion in the U.S.

While vericiguat did meet the primary compositive endpoint of CVD or first hospitalization for heart failure in the pivotal VICTORIA trial, vericiguat did not show statistical superiority vs. placebo for the secondary endpoints of CVD or all-cause mortality. Compared indirectly to Entresto and the sodium glucose co-transporter 2 (SGLT2) inhibitor Farxiga® (dapagliflozin), the results for vericiguat appear less robust, but comparing across different trials is challenging and the VICTORIA trial did include a more high-risk heart failure population. Entresto was approved for heart failure in 2015 and Farxiga received an indication for heart failure in May 2020.

Given the results of the pivotal trial and the availability of other treatment options that have been on the market for longer, vericiguat may be used further down in the treatment algorithm, with initial use potentially limited to high-risk or symptomatic patients or patients who experience adverse events with existing therapies. In addition, while a subset of patients in the trial were also receiving background therapy with Entresto, the sample size was small and the additive benefit of vericiguat in these patients is unknown.

For reference, the WAC for Entresto is approximately $6,600 per year.

- Advantages: Novel MOA, unmet need, large potential target population, oral and once daily
- Disadvantages: Alternatives available (eg, Entresto, SGLT2 inhibitors), Lack of robust data for CVD and all-cause mortality benefit
- Reference WAC (Entresto): ~$6,600 per year

Voclosporin (Brand Name: To be determined)

Manufacturer: Aurinia Pharmaceuticals
Regulatory designations: Fast Track
Expected FDA decision: 1/22/2021

Therapeutic use

Voclosporin is in development for the treatment of lupus nephritis.

Systemic lupus erythematosus (SLE) is an autoimmune disease that can affect different tissues and organs in the body. Lupus nephritis is an inflammatory disease of the kidneys caused by SLE. Of the approximately 500,000 people in the U.S. with SLE, about 50% will develop lupus nephritis. Between 10 to 30% of people with lupus nephritis develop kidney failure.

- Treatment of lupus nephritis

Clinical profile

Voclosporin is a calcineurin inhibitor. By inhibiting calcineurin, voclosporin blocks interleukin (IL)-2 expression and T-cell mediated immune responses and stabilizes the kidney. Compared to other calcineurin inhibitors (eg, cyclosporine), voclosporin is expected to have a more predictable pharmacokinetic and pharmacodynamic relationship and increased potency.

- Calcineurin inhibitor
- Oral formulation
- Renal response: 40.8% vs. 22.5% with placebo
- Common AEs: Infections, gastrointestinal disorders
- Dosing: Twice daily

Voclosporin (continued...)

Pivotal trial data:
The efficacy of voclosporin was evaluated in AURORA, a Phase 3, randomized, double-blind, placebo-controlled study in 357 patients with lupus nephritis. Patients were randomized to voclosporin or placebo, and all patients received background therapy of mycophenolate mofetil and low dose steroids. The primary endpoint was complete renal response at 52 weeks. Renal response was defined as: (1) urinary protein-to-creatinine ratio (UCPR) of ≤ 0.5 mg/mg, (2) estimated glomerular filtration rate (eGFR) ≥ 60 mL/min/1.73 m2, or no confirmed decrease from baseline in eGFR of > 20%, (3) presence of sustained, low dose steroids and (4) no administration of rescue medication. The renal response rates were 40.8% and 22.5% for voclosporin and placebo, respectfully ($p < 0.001$).

In addition, the efficacy of voclosporin was evaluated in AURA-LV, a Phase 2, dose-ranging, randomized, double-blind, placebo-controlled trial in 265 patients with lupus nephritis. Patients received a low-dose or high-dose of voclosporin or placebo, in combination with mycophenolate mofetil and low-dose oral corticosteroids. The primary endpoint and key secondary endpoint were complete renal remission at week 24 and week 48, respectively. At week 24 complete renal remission was achieved by 32.6% of patients in the low-dose voclosporin group ($p = 0.046$ vs. placebo), 27.3% of patients in the high-dose voclosporin group ($p = 0.204$ vs. placebo), and 19.3% of patients in the placebo group. Both low-dose and high-dose voclosporin were superior to placebo at week 48 with remission achieved by 49.4% of patients in the low-dose voclosporin group ($p < 0.001$) and 39.8% of patients in the high-dose voclosporin group ($p = 0.026$) vs. 23.9% in the placebo group.

Safety:
The safety data for voclosporin from the Phase 3 trial are limited but rates of serious adverse events were similar between the voclosporin and the control arms.

In the Phase 2 trial, the most common adverse events were infections and gastrointestinal disorders.

Dosing:
In the pivotal trials, voclosporin was administered orally twice daily.

- Calcineurin inhibitor
- Oral formulation
- Renal response: 40.8% vs. 22.5% with placebo
- Common AEs: Infections, gastrointestinal disorders
- Dosing: Twice daily

Voclosporin (continued...)

Competitive environment

Voclosporin would potentially be the first FDA approved treatment specifically for lupus nephritis. The current standard of care includes induction therapy with corticosteroids plus immunosuppressants (eg, cyclophosphamide or mycophenolate mofetil) followed by maintenance treatment with mycophenolate mofetil or azathioprine. These agents have been used off-label for many years but up to 30% of patients still end up with end-stage renal disease (ESRD), particularly patients who are either partial or non-responders to existing treatment options.

The results from the pivotal trial were promising with voclosporin providing a significant improvement in renal response vs. the current standard of care. In addition, while structurally similar to other calcineurin inhibitors (eg, cyclosporine), voclosporin is more potent and is not expected to require strict monitoring of drug levels. Based on the limited safety data available, voclosporin appeared to be relatively well tolerated but more information is needed as other calcineurin inhibitors can cause kidney dysfunction, hyperkalemia, diabetes, and an increase in blood pressure. The Phase 2 trial did find an imbalance in mortality with more deaths reported in the low dose voclosporin group, but this was not replicated in the Phase 3 trial.

While there are currently no FDA approved treatments for lupus nephritis, GlaxoSmithKline's injectable B-lymphocyte stimulator-specific inhibitor, Benlysta® (belimumab), has been approved for SLE since 2011 and is being currently evaluated by the FDA for lupus nephritis; the FDA is expected to make a decision for Benlysta's new indication in the first half of 2021.

For reference, the WAC for subcutaneously (SC) administered Benlysta is approximately $49,300 per year.

- Advantages: Potentially first FDA approved product for lupus nephritis, promising trial results, unmet need, oral administration
- Disadvantages: Requires continued use with other immunosuppressants and corticosteroids, limited safety data, potential future competition with GlaxoSmithKline's Benlysta
- Reference WAC (Benlysta): ~$49,300 per year

Pegunigalsidase alfa (Brand Name: To be determined)

Manufacturer: Protalix
Regulatory designations: Fast Track
Expected FDA decision: 1/27/2021

Therapeutic use

Pegunigalsidase alfa is in development for the treatment of adult patients with Fabry disease.

Fabry disease is a rare, X-linked inherited disorder of glycosphingolipid metabolism resulting from the absent or markedly deficient activity of the lysosomal enzyme, α-galactosidase A (α-Gal A). The enzyme deficiency causes a build-up of globotriaosylceramide (Gb3) and related glycolipids in the body's cells, resulting in the cell abnormalities and organ dysfunction that particularly affect small blood vessels, the heart and kidneys. In adults, cardiac (eg, left ventricular hypertrophy, heart failure, coronary artery disease) and cerebrovascular involvement (transient ischemic attacks and ischemic strokes) accounts for the majority of deaths associated with Fabry disease. Patients can also develop ESRD.

Fabry disease occurs in one person per 40,000 to 60,000.

- Treatment of adult patients with Fabry disease

Clinical profile

Pegunigalsidase alfa is a plant-cell based, chemically modified stabilized version of the recombinant α-Gal A enzyme (enzyme replacement therapy). Compared to mammalian cell-based production, plant-cell based production does not carry the risk of infection by human or animal pathogens which reduces the costs associated with developing these therapies (eg, less costly maintenance).

- Enzyme replacement therapy
- IV formulation
- Improvement in renal function (as measured by the mean annualized eGFR slope) vs. Replagal
- Limited safety data
- Dosing: Once every 2 weeks

Pegunigalsidase alfa (continued...)

Pivotal trial data:
The efficacy of pegunigalsidase alfa was also evaluated in the BRIDGE trial, a Phase 3, open-label, single arm, switch-over study in 22 patients with Fabry disease previously treated with Replagal® (agalsidase alfa) (enzyme replacement therapy approved in Europe). Patients in the BRIDGE study were screened and evaluated over 3 months while continuing Replagal treatment. Following the screening period, each patient was enrolled and switched from Replagal to pegunigalsidase alfa for 12 months.

Topline results of the data in the study showed improvement in renal function as measured by mean annualized estimated glomerular filtration rate (eGFR) slope in patients who were switched from Replagal to pegunigalsidase alfa. In the study, the mean annualized eGFR slope of the study participants improved from -5.90 mL/min/1.73m2/year while on Replagal to -1.19 mL/min/1.73m2/year on pegunigalsidase alfa.

Safety:
The safety data for pegunigalsidase alfa are limited but in the Phase 3 trial it was reported that all adverse events were transient in nature without sequelae.

Dosing:
In the pivotal trials, pegunigalsidase alfa was administered via IV infusion every 2 weeks.

Competitive environment

Pegunigalsidase alfa would provide an additional option in the treatment of Fabry disease. In the U.S., pegunigalsidase alfa would be competing directly with Sanofi's enzyme replacement therapy, Fabrazyme® (agalsidase beta). Due to its unique molecular structure, pegunigalsidase alfa has a longer half-life than Fabrazyme and is expected to have improved immunogenicity (fewer cases of anti-drug antibodies). While the efficacy data currently submitted to the FDA is based on every 2-week dosing, Protalix is also evaluating a every 4-week regimen which would reduce the number of injections vs. Fabrazyme (dosed every 2 weeks). A head-to-head trial (BALANCE) vs. Fabrazyme is ongoing with data expected in the first half of 2021 and another switch trial (from Fabrazyme or Replagal) is expected to report out data in the first quarter of 2021.

In addition to Fabrazyme, pegunigalsidase alfa will also be competing with Amicus' oral Galafold® (migalastat), an α-Gal A pharmacological chaperone which can be used in patients with amenable mutations (35% to 50% of the overall Fabry disease population).

For reference, the WAC for Fabrazyme is approximately $290,000 per year.

- Advantages: Longer half-life and improved immunogenicity vs. Fabrazyme, potential improvement in clinical outcomes vs. current standard of care
- Disadvantages: Alternatives available (Fabrazyme and Galafold), lack of head-to-head trial results, IV administration
- Reference WAC (Fabrazyme): ~$290,000 per year

Evinacumab (Brand Name: To be determined)

Manufacturer: Regeneron
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 2/11/2021

Therapeutic use

Evinacumab is in development as an adjunct to other lipid-lowering therapies in patients with homozygous familial hypercholesterolemia (HoFH).

HoFH is an ultra-rare inherited disease that causes severely elevated levels of low-density lipoprotein cholesterol (LDL-C). HoFH is most often caused by the presence of loss-of-function variants in the LDL receptor, which leads to low or zero clearance of LDL-C from the circulation. Patients with HoFH are at increased risk of severe vascular disease by the teenage years. Without aggressive treatment, including LDL-C apheresis and HoFH specific medications, mortality is common before age 30.

HoFH affects approximately 1 in 300,000 people and Regeneron estimates that there are 1,300 patients in the U.S. with the disease.

- Adjunct to other lipid-lowering therapies in patients with HoFH

Clinical profile

Evinacumab is a human monoclonal antibody targeting angiopoietin-like 3 (ANGPTL3). ANGPTL3 is an inhibitor of lipoprotein and endothelial lipase and plays a key role in lipid metabolism by increasing the levels of triglycerides and other lipids.

Loss-of-function variants in ANGPTL3 have been associated with low levels of LDL-C and with a lowered risk of coronary artery disease. Both ANGPLT3 loss-of-function variants and ANGPTL3 pharmacologic inhibition reduce LDL-C levels independently of the LDL receptor.

- Monoclonal antibody targeting ANGPTL3
- IV formulation
- Change in LDL-C at week 24: reduction of 47.1% vs. increase of 1.9% with placebo
- Common AEs: Influenza-like illness, rhinorrhea
- Dosing: Every 4 weeks

Evinacumab (continued...)

Pivotal trial data:
The efficacy of evinacumab was evaluated in ELIPSE, a Phase 3, randomized, double-blind, placebo-controlled trial in 65 patients with HoFH who were receiving stable lipid-lowering therapy. Patients were randomized to evinacumab or placebo. The primary outcome was the percent change from baseline in the LDL-C level at week 24.

The mean baseline LDL-C in the evinacumab and placebo groups was 255.1 mg/dL. At week 24, patients receiving evinacumab had a relative reduction from baseline in LDL-C of 47.1% vs. an increase of 1.9% in the placebo group, for a between-group least-squares mean (LSM) difference of -49.0 percentage points (95% CI: -65.0, -33.1; $p < 0.001$). The between-group LSM absolute difference in the LDL-C level was -132.1 mg/dL (95% CI: -175.3, -88.9; $p < 0.001$).

Safety:
The most common adverse events with evinacumab use were influenza-like illness and rhinorrhea.

Dosing:
In the pivotal trial, evinacumab was administered via IV infusion every 4 weeks.

Competitive environment

Evinacumab would offer an additional treatment option and provides a novel mechanism of action (MOA) for the treatment of HoFH. In the pivotal trial, evinacumab was well tolerated and provided substantial reductions in LDL-C, including in patients typically very difficult to treat (null–null homozygosity HoFH).

The current pharmacological standard of care includes statins and proprotein convertase subtilisin–kexin type 9 (PCSK9) inhibitors (eg, Repatha® [evolocumab]). Orally administered Juxtapid® (lomitapide) is also approved for HoFH but its use has been limited due to poor tolerability. While these medications are effective in lowering LDL-C, most HoFH patients still do not reach LDL-C target goals. SC administered PCSK9 inhibitors can provide significant reductions in the general HoFH population but have poor efficacy in difficult to treat HoFH patients.

Compared to orally administered lipid-lowering agents and PCSK9 inhibitors, the target population for evinacumab is expected to be much smaller since the initial indication is specifically for HoFH. Additionally, statins are available generically and because PCSK9 inhibitors have broader indications, they are not priced like orphan drugs. Evinacumab will likely have a higher cost relative to PCSK9 inhibitors due to the narrow indication and it does require monthly IV administration.

- Advantages: Novel MOA for the treatment of HoFH, substantial reductions in LDL-C in difficult to treat HoFH patients, well tolerated
- Disadvantages: Alternatives available (including PCSK9 inhibitors), small initial target population, monthly IV administration

Umbralisib (Brand Name: To be determined)

Manufacturer: TG Therapeutics
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 2/15/2021 (marginal zone lymphoma) and 6/15/2021 (follicular lymphoma)

Therapeutic use

Umbralisib is in development for treatment of patients with previously treated marginal zone lymphoma (MZL) who have received at least one prior anti-CD20 based regimen. Umbralisib is also seeking approval for a second indication for patients with follicular lymphoma (FL) who have received at least two prior systemic therapies.

MZL comprises a group of indolent (slow growing) mature B-cell non-Hodgkin lymphomas (NHLs). MZL is generally considered a chronic and incurable disease. MZL is the third most common B-cell NHL, accounting for approximately 8% of all NHL cases. The annual incidence of MZL is approximately 7,500 newly diagnosed patients in the U.S.

FL is typically an indolent form of NHL that arises from B-lymphocytes. Similar to MZL, FL is generally not curable and is considered a chronic disease. It is the second most common form of NHL with an annual incidence in the U.S. of approximately 15,000 newly diagnosed patients.

- Treatment of patients with previously treated MZL who have received at least one prior anti-CD20 based regimen and patients with FL who have received at least two prior systemic therapies

Clinical profile

Umbralisib is a dual inhibitor of phosphoinositide-3-kinase (PI3K) delta and casein kinase 1 (CK1) epsilon. PI3Ks are a family of enzymes involved in various cellular functions, including cell proliferation and survival, cell differentiation, intracellular trafficking, and immunity. Inhibition of CK1 epsilon is believed to have direct anti-cancer effects and may also modulate T-cell activity associated with immune-mediated adverse events seen with previous PI3K inhibitors.

- PI3K delta and CK1 epsilon inhibitor
- Oral formulation
- ORR (MZL): 52%
- ORR (FL): 40% to 50%
- Common AEs: Diarrhea, nausea, fatigue, neutropenia, diarrhea, increased ALT/AST
- Dosing: Once daily

<u>Pivotal trial data:</u>
The FDA filing for umbralisib was based primarily on data from the umbralisib monotherapy MZL and FL cohorts of the UNITY-NHL Phase 2b, open-label trial. The MZL cohort evaluated the efficacy of single agent umbralisib in 42 patients with MZL who have received at least one prior anti-CD20 regimen. Based on an analysis of the interim MZL efficacy population with a median follow-up of 12.5 months, the ORR was 52%. The Kaplan-Meier (KM) estimate of PFS at 12 months was 66%, with the median PFS not reached.

The FL cohort evaluated single agent umbralisib in 118 patients with FL who were relapsed or refractory following at least two prior lines of therapy, including an anti-CD20 regimen and an alkylating agent. In October 2019, TG Therapeutics announced that umbralisib met the prespecified ORR target of 40% to 50%.

<u>Safety:</u>
The most common adverse events with umbralisib use were diarrhea, nausea, fatigue, neutropenia, and increased alanine aminotransferase (ALT)/aspartate aminotransferase (AST).

<u>Dosing:</u>
In the pivotal trial, umbralisib was administered orally once daily.

Umbralisib (continued...)

Competitive environment

Umbralisib would be the first PI3K inhibitor approved for MZL. In the relapsed or refractory MZL setting, patients have limited treatment options and the only drug approved is the Bruton's tyrosine kinase (BTK) inhibitor, Imbruvica® (ibrutinib). Compared indirectly, umbralisib does appear to provide slightly better complete response rates vs. Imbruvica in this population.

For FL, umbralisib would be entering a crowded marketplace and it would be competing with two other PI3K inhibitors, Zydelig® (idelalisib) and Copiktra® (duvelisib). The response rate with umbralisib appears to be comparable to Zydelig and Copiktra, but umbralisib's unique dual MOA and selectivity for PI3K delta may provide an advantage from a tolerability standpoint. Zydelig and Copiktra have boxed warnings for diarrhea and colon inflammation as well as pneumonitis. From a dosing perspective, umbralisib also offers an advantage as its dosed once daily vs. Zydelig and Copiktra which are dosed twice daily.

While umbralisib may provide improved tolerability vs. other PI3K inhibitors, limited data is currently available for both the efficacy and safety of umbralisib and the FDA is currently reviewing the drug under the accelerated approval pathway.

Finally, umbralisib, as part of a combination regimen, is also currently in development for other hematologic malignancies, including chronic lymphocytic leukemia (CLL) which could expand its future use.

For reference, the WAC for Copiktra is approximately $13,275 per 30 days.

- Advantages: Potentially the first PI3K inhibitor for MZL, may provide better tolerability vs. other drugs in class, oral and once daily, potential future use in other hematologic malignancies
- Disadvantages: Alternatives available for FL, limited trial data, narrow initial indication
- Reference WAC (Copiktra): ~$13,275 per 30 days

Casimersen (Brand Name: Amondys 45™)

Manufacturer: Sarepta
Regulatory designations: Orphan Drug
Expected FDA decision: 2/25/2021

Therapeutic use

Casimersen is in development for the treatment of patients with Duchenne muscular dystrophy (DMD) who have genetic mutations that are amenable to skipping exon 45 of the dystrophin gene.

DMD is a rare genetic disorder characterized by progressive muscle deterioration and weakness. It is an X-linked disorder that affects young boys with a prevalence of approximately 1 in every 3,500 live male births. There are an estimated 6,000 males affected with DMD in the U.S. About 8% of those patients have mutations amenable to exon 45 skipping.

DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. The onset of symptoms occurs between 3 and 5 years of age and worsens over time. Progressive muscle weakness leads to decreased ambulation, inability to perform activities independently and confinement to a wheelchair by the early teen age years. Later, patients experience life-threatening heart and respiratory conditions, with death commonly occurring in the late teens or twenties.

- Treatment of patients with DMD who have genetic mutations that are amenable to skipping exon 45 of the dystrophin gene

Clinical profile

Casimersen is a phosphorodiamidate morpholino oligomer. It is designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to allow for production of an internally truncated, yet functional, dystrophin protein.

- Exon-skipping phosphorodiamidate morpholino oligomer
- IV formulation
- Mean dystrophin protein: Increased to 1.736% of normal compared to a mean baseline of 0.925% of normal
- Limited safety data
- Dosing: Once weekly

Casimersen (Brand Name: Amondys 45™) (continued...)

Pivotal trial data:

The efficacy of casimersen was evaluated in the ESSENSE trial, a Phase 3, randomized, double blind, placebo-controlled study in DMD patients. Patients with mutations amenable to exon 45 or 53 skipping were randomized to receive casimersen or Vyondys 53® (golodirsen), respectively, or placebo for up to 96 weeks. A total of 27 patients received casimersen vs. 16 patients with placebo.

An interim analysis was performed on data from biopsies of the bicep muscle at baseline and on-treatment at week 48. In the casimersen arm, mean dystrophin protein (% normal dystrophin as measured by western blot) increased to 1.736% of normal compared to a mean baseline of 0.925% of normal ($p < 0.001$). A statistically significant difference in the mean change from baseline to week 48 in dystrophin protein was observed between casimersen and placebo ($p = 0.009$).

Safety:

To date, safety data has not been published or announced by Sarepta.

Dosing:

In the pivotal trial, casimersen was administered via IV infusion once weekly.

Competitive environment

If approved, casimersen would be the first approved drug for DMD patients with mutations amenable to exon 45 skipping and there is a high unmet need given the severity of the condition.

However, similar to Sarepta's other exon skipping agents, the FDA submission for casimersen is based on limited data demonstrating a very modest improvement in a surrogate endpoint (dystrophin levels). The clinical significance of a small change in dystrophin has not been established. The efficacy of casimersen is similar to Exondys 51® (eteplirsen) and Vyondys 53, Sarepta's other DMD products which are used in patients with mutations amenable to exon 51 and 53 skipping, respectively.

The expected target population for casimersen is small given the rare nature of DMD and because only 8% of patients with DMD have a mutation amendable to exon 45 skipping. Like other exon skipping agents, casimersen requires weekly IV infusion.

For reference, the WAC for Vyondys 53 is approximately $748,800 per year (for a patient weighing 30 kg), but the cost can vary significantly patient-to-patient due to weight-based dosing.

- Advantages: Potentially the first approved drug for exon 45 skipping, high unmet need
- Disadvantages: Efficacy measured using surrogate endpoint of dystrophin improvement (very modest benefit demonstrated), small eligible patient population, IV administration
- Reference WAC (Vyondys 53): ~$748,000 (for a 30 kg patient) per year (cost can vary due to weight-based dosing)

Aducanumab (Brand Name: To be determined)

Manufacturer: Biogen
Regulatory designations: Fast Track
Expected FDA decision: 3/7/2021

Therapeutic use

- Treatment for Alzheimer's disease

Aducanumab is in development for the treatment for Alzheimer's disease.

Alzheimer's disease is an irreversible, progressive brain disorder that slowly destroys memory and cognition. The disease is characterized by changes in the brain, including the abnormal accumulation of toxic amyloid beta plaque.

Alzheimer's is the most common cause of dementia among older adults with an estimated 5.7 million Americans living with the disease in 2018. It is the fifth leading cause of death for adults aged 65 years and older, and the sixth leading cause of death for all adults.

Clinical profile

- Beta amyloid-targeting monoclonal antibody
- IV formulation
- EMERGE: Statistically significant reduction from baseline in CDR-SB scores at 78 weeks for high dose aducanumab (difference vs. placebo: -0.39)
- ENGAGE: No statistically significant difference vs. placebo in CDR-SB scores at 78 weeks
- Common AEs: ARIA-E and ARIA-H; headache, dizziness, visual disturbances, nausea, vomiting
- Dosing: Once monthly

Aducanumab is a human monoclonal antibody that selectively binds to amyloid beta fibrils and soluble oligomers and reduces amyloid plaques in the brain.

Pivotal trial data:
The efficacy of aducanumab was evaluated in two Phase 3, randomized, double blind, placebo-controlled studies (EMERGE and ENGAGE) in patients with mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease. Patients were randomized to a low or high dose of aducanumab or placebo. The primary endpoint was cognitive and functional impairment as measured by changes in the Clinical Dementia Rating-Sum of Boxes (CDR-SB) score. The CDR is an evaluation of a patient's cognitive status across 6 domains of functioning including memory, orientation, judgment and problem solving, community affairs, home and hobbies, and personal care. The CDR-SB score is obtained by summing each of the domain box scores, with scores ranging from 0 to 18. A key secondary efficacy endpoint was clinical decline as measured by the Mini-Mental State Examination (MMSE). The MMSE is a common assessment tool for patients with Alzheimer's disease with a maximum score of 30 and with lower scores indicating more severe cognitive problems.

In March 2019, Biogen announced that both pivotal trials were stopped based on results of a futility analysis conducted by an independent data monitoring committee, which indicated the trials were unlikely to meet their primary endpoint upon completion. However, in October 2019, Biogen announced that they would be pursuing an FDA filing based on a new analysis, conducted by Biogen in consultation with the FDA, of a larger dataset from the pivotal trials. This new analysis of a larger dataset included additional data that became available after the pre-specified futility analysis.

In the final data set for the EMERGE trial (N = 1,638), patients treated with high dose aducanumab showed a statistically significant reduction of clinical decline from baseline in CDR-SB scores at 78 weeks (difference vs. placebo of -0.39; p = 0.0120). There were also statistically significant improvements in key secondary endpoints including MMSE (difference vs. placebo of 0.6, p = 0.0493). The low dose aducanumab arm did not demonstrate statistically significant improvements in either the primary or secondary endpoints vs. placebo.

In the ENGAGE trial (N = 1,647), neither the low nor high dose aducanumab arms showed statistically significant improvements vs. placebo for any of the efficacy endpoints vs. placebo. In contrast to EMERGE, patients in the high dose aducanumab arm had numerical worsening for the CDR-SB and MMSE endpoints.

Aducanumab (continued...)

Safety:
The most common adverse events with aducanumab use were amyloid-related imaging abnormalities-edema (ARIA-E) and amyloid related imaging abnormality-micro-hemorrhages (ARIA-H).

ARIA-related adverse events represent a spectrum of findings detected on brain imaging. The exact mechanism is not understood but published hypotheses suggest that they occur due to increased cerebrovascular permeability. Symptoms reported in patients with ARIA included headache, dizziness, visual disturbances, nausea, and vomiting.

Dosing:
In the pivotal trials, aducanumab was administered via IV infusion once monthly.

Competitive environment

If approved, aducanumab would potentially be the first disease modifying therapy for Alzheimer's disease. There is a high unmet need for treatments since Alzheimer's is a leading cause of morbidity and mortality among the elderly and there is a lack of treatment options that have shown benefit in reducing or slowing cognitive decline. The estimated target population is also substantial as Alzheimer's disease affects over 5 million people in the U.S. and Biogen estimates that about 1.4 million would be eligible for therapy with aducanumab based on mild disease status and confirmed presence of amyloid beta. Cholinesterase inhibitors (eg, Aricept® [donepezil], Exelon® [rivastigmine]) and the NMDA inhibitor Namenda® (memantine) are the only currently available symptomatic medications for cognition in patients with Alzheimer's disease but these drugs have limited benefit and are considered symptomatic therapies that do not change the underlying pathophysiology of the disease.

In clinical trials, patients treated with aducanumab have substantial reductions of amyloid plaques in the brain which is consistent with the MOA for the drug. However, despite these changes in brain imaging, the clinical outcomes for aducanumab were highly questionable. Even based on the re-analysis of the pivotal trial data, aducanumab only demonstrated a statistically significant improvement in the efficacy endpoints in one of the two pivotal trials. In the one positive trial, the improvements in the cognitive measures were small in magnitude and generally less than what would be considered clinically meaningful. The results of these trials as well other investigational therapies have raised questions about whether amyloid is the correct target for Alzheimer's disease treatments.

In addition to the questionable efficacy, aducanumab was also associated with ARIA-related side effects, including edema and microhemorrhages which would require additional provider monitoring and potentially APOE ε4 allele gene testing (carriers are at higher risk for developing ARIA). Finally, unlike the currently available oral symptomatic therapies for Alzheimer's disease, aducanumab requires monthly IV infusions.

On November 6, 2020, an FDA Advisory Committee was convened to discuss the safety and efficacy of aducanumab. The Committee did not endorse aducanumab and of the 11 voting members, 8 voted that they did not think the EMERGE trial provided strong evidence that supports the effectiveness for the treatment of Alzheimer's disease (1 voted yes and 2 abstained).

- Advantages: Potentially the first approved disease modifying therapy for Alzheimer's disease, significant unmet need
- Disadvantages: Questionable clinical efficacy, safety concerns including ARIA-E and ARIA-H, requires monthly IV infusions

Arimoclomol (Brand Name: To be determined)

Manufacturer: Orphazyme
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
Expected FDA decision: 3/17/2021

Therapeutic use

Arimoclomol is in development for the treatment of Niemann-Pick disease type C.

Niemann-Pick disease type C is an ultra-rare genetic disorder characterized by an inability of the body to transport cholesterol and lipids inside of cells. This leads to the abnormal accumulation of these substances within various parts of the body, including the brain, lungs, liver, and spleen. The disease can range from a fatal disorder within the first few months after birth to a late onset, chronic progressive disorder that remains undiagnosed well into adulthood. Symptoms or complications can include difficulty coordinating movements, poor muscle tone (dystonia), severe liver disease, and interstitial lung disease. Patients can also develop problems with speech and swallowing that worsen over time. Death typically occurs from aspiration pneumonia in the second or third decade of life.

Niemann-Pick disease type C is estimated to occur in 1 in 100,000 to 120,000 live births. Orphazyme estimates 300 patients are currently diagnosed with the disease in the U.S.

Clinical profile

Arimoclomol has been shown to increase the production of cell protective heat shock proteins (HSPs). HSPs are a family of proteins whose levels are amplified by cells in response to exposure to a wide variety of stressful conditions. HSPs promote the survival of stressed cells by ensuring correct folding and function of misfolded proteins.

The increase in the production of naturally occurring HSPs inside the cells, reduces protein misfolding and aggregation and is believed to improve lysosomal function.

- Treatment of Niemann-Pick disease type C

- HSP amplifier
- Oral formulation
- Mean change from baseline in the NPC-CSS: 1.9 vs. 0.5 with placebo (not statistically significant)
- Response rate (CGI-I scale): 56.3% vs. 58.8% with placebo (not statistically significant)
- Limited safety data
- Dosing: Three times a day

Arimoclomol (continued...)

Pivotal trial data:

The efficacy of arimoclomol was evaluated in a Phase 2/3, randomized, double blind, placebo-controlled study in 50 patients (between the age of 2 to 18 years) with Niemann-Pick disease type C. Patients received arimoclomol or placebo, in addition to the patient's routine clinical care. The primary endpoints were the change in the 5-domain NPC Clinical Severity Scale (NPC-CSS) (ambulation, fine motor skills, swallowing, speech and cognition) and response rates, as measured by the Clinical Global Impression of Improvement scale (CGI-I).

After 12 months, there was a directional improvement in the 5-domain NPC-CSS in favor of arimoclomol, but the difference was not statistically significant vs. placebo (mean change from baseline: 1.9 vs. 0.5; p = 0.0506). In the predefined subgroups of patients 4 years and older (44 out of 50 randomized patients in the trial) and in patients receiving Zavesca® (miglustat) as a part of the routine clinical care, the difference was statistically significant. No difference was observed between the two groups for the CGI-I scale; however, in patients who severely progressed during the trial, only 10.7% of the arimoclomol-treated patients got 'much worse' or 'very much worse' vs. 26.7% in the placebo control group.

Safety:

Safety data for arimoclomol is limited but the overall incidence of adverse events was similar for arimoclomol (85.7%) and placebo (81.3%).

Dosing:

In the pivotal trial, arimoclomol was administered orally three times daily.

Competitive environment

If approved, arimoclomol would be the first FDA approved treatment for Niemann-Pick disease type C. There is a high unmet need for treatments as the disease is debilitating and associated with increased risk of mortality. The current standard of care is supportive care for symptoms. Zavesca, an oral agent approved for Gaucher disease, has been used off-label for Niemann-Pick disease type C based on limited and conflicting evidence.

The data from the pivotal Phase 2/3 trial were underwhelming as arimoclomol failed to achieve statistically significant improvements for either of the co-primary endpoints vs. placebo. An ongoing extension trial is evaluating the potential long-term benefits of arimoclomol in delaying or preventing the progression of the disease. Limited positive interim data was announced from the extension trial, but additional information, particularly specific numerical improvements, are needed to assess the clinical meaningfulness of the results.

The initial target population for arimoclomol is expected to be very small given the rarity of Niemann-Pick disease type C but arimoclomol is also being evaluated for the treatment of several other orphan diseases, including amyotrophic lateral sclerosis (ALS), sporadic inclusion body myositis, and Gaucher disease.

The projected WAC for arimoclomol is between $300,000 to $600,000 per year.

- Advantages: Potentially the first FDA approved drug for Niemann-Pick disease type C, high unmet need, potential future use in other conditions (eg, ALS)
- Disadvantages: Lack of robust efficacy data, small initial target population
- Projected WAC: $300,000 to $600,000

Ponesimod (Brand Name: To be determined)

Manufacturer: Janssen
Expected FDA decision: 3/18/2021

Therapeutic use

Ponesimod is in development for the treatment of adult patients with relapsing multiple sclerosis (MS).

MS is a chronic disorder of the central nervous system (CNS). MS typically starts with a relapsing-remitting course, in which episodes of worsening function (relapses) are followed by recovery periods (remissions). These remissions may not be complete and may leave patients with some degree of residual disability.

The overall estimated prevalence of MS in the U.S. may be as high as 1 million individuals and about 85% of patients are initially diagnosed with a relapsing form of MS.

- Treatment of adult patients with relapsing MS

Clinical profile

Ponesimod is a selective sphingosine-1-phosphate receptor 1 (S1P1) modulator that inhibits S1P protein activity and is believed to reduce the number of circulating lymphocytes that can cross the blood-brain barrier.

In patients with MS, the movement of immune cells into the brain damages myelin, the protective sheath that insulates nerve cells. Damage to myelin slows or halts nerve conduction, producing the neurologic signs and symptoms of MS.

- S1P1 modulator
- Oral formulation
- ARR: 30.5% reduction up to week 108 vs. Aubagio
- Common AEs: Nasopharyngitis, headache, upper respiratory tract infections, increased ALT
- Dosing: Once daily

<u>Pivotal trial data:</u>
The efficacy of ponesimod was evaluated in OPTIMUM, a Phase 3, randomized, double-blind, active-controlled study in 1,133 patients with MS. Patients were randomized to receive ponesimod or Aubagio® (teriflunomide), another approved oral MS drug. The primary endpoint was measured by annualized relapse rate (ARR) from baseline to end of study. ARR was defined as the number of confirmed relapses per subject-year.

The ARR was 0.202 vs. 0.290 for ponesimod and Aubagio, respectively (30.5% reduction up to week 108; $p = 0.0003$).

<u>Safety:</u>
The most common adverse events with ponesimod use were nasopharyngitis, headache, upper respiratory tract infections, and increased ALT.

<u>Dosing:</u>
In the pivotal trial, ponesimod was administered orally once daily.

Ponesimod (continued...)

Competitive environment

If approved, ponesimod would offer an additional selective S1P receptor modulator for the treatment of MS and can be dosed orally once a day.

However, there are many oral and injectable alternative products for relapsing forms of MS, including drugs in the same class. Ponesimod would be a relatively late market entry – Novartis' non-selective S1P modulator, Gilenya® (fingolimod), has been available since 2010, and their selective S1P modulator, Mayzent® (siponimod), was launched in March 2019. Celgene's selective S1P modulator Zeposia® (ozanimod) was approved in March 2020.

Additionally, ponesimod was not compared head-to-head against the other S1P modulators. Compared indirectly, it does not appear that ponesimod provides better ARR reductions vs. drugs in the same class.

For reference, the WAC for Zeposia is approximately $86,000 per year.

- Advantages: Additional oral selective S1P receptor modulator
- Disadvantages: Alternatives available, late market entry, lack of head-to-head data vs. other S1P modulators
- Reference WAC (Zeposia): ~$86,000 per year

Idecabtagene vicleucel (Brand Name: To be determined)

Manufacturer: Bristol-Myers Squibb/bluebird bio
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 3/27/2021

Therapeutic use

Idecabtagene vicleucel is in development for the treatment of adult patients with multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody.

- Treatment of adult patients with multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody

Multiple myeloma is a cancer of the plasma cells (white blood cells that produce antibodies). Multiple myeloma is a relatively uncommon cancer with a lifetime risk of 1 in 132 (0.76%). In the U.S., about 32,270 new cases are estimated in 2020 and about 12,830 deaths are expected to occur.

While many treatment options are available for multiple myeloma, almost all patients with multiple myeloma who survive initial treatment will eventually relapse and require further therapy. The 5-year survival rate is approximately 50%.

Clinical profile

Idecabtagene vicleucel is a B-cell maturation antigen (BCMA)-directed genetically modified autologous chimeric antigen receptor (CAR) T cell immunotherapy.

- BCMA-targeted CAR T cell therapy
- IV formulation
- ORR: 73% across all dose levels
- Common AEs: Cytopenia and CRS
- Dosing: One-time dose

BCMA promotes plasma cell survival and BCMA is expressed at varying levels in myeloma patients. Idecabtagene vicleucel binds to BCMA on the surface of multiple myeloma cells leading to CAR T cell proliferation, cytokine secretion, and subsequent cytolytic killing of BCMA-expressing cells.

Pivotal trial data:
The efficacy of idecabtagene vicleucel was evaluated in KarMMa, a Phase 2, open-label, single-arm study in 128 adults with relapsed and refractory multiple myeloma. The primary endpoint of the study was ORR.

The ORR was 73% across all dose levels of idecabtagene vicleucel, including 33% of patients who had a complete response. Median DOR was 10.7 months. Median PFS was 8.8 months. As of May 2020, the OS data were continuing to mature, with an estimated median OS of 19.4 months across all dose levels and 78% of patients alive at 12 months.

Safety:
The most common adverse events with idecabtagene vicleucel use were cytopenia and cytokine release syndrome (CRS).

Dosing:
In the pivotal trial, idecabtagene vicleucel was administered as a one-time IV infusion.

Idecabtagene vicleucel (continued...)

Competitive environment

If approved, idecabtagene vicleucel would become the first CAR T cell therapy for multiple myeloma, offering an additional treatment option for patients who have failed previous treatment options. It is administered as a one-time dose.

The data currently available for idecabtagene vicleucel are promising with response rates that when compared indirectly, are higher in this setting of multiple myeloma. However, there is a lack of long-term remission data for CAR T cell therapies, which is important considering the high cost of the one-time administration. Idecabtagene vicleucel will be competing against several other FDA-approved drugs for relapsed/refractory multiple myeloma. This includes another recently approved anti-BCMA therapy, GlaxoSmithKline's monoclonal antibody Blenrep® (belantamab mafodotin-blmf).

Like other FDA-approved CAR T cell therapies, idecabtagene vicleucel could potentially receive a boxed warning and REMS program for CRS, which would require close monitoring post-administration. Furthermore, treatment delays may also occur due to the long preparation process needed to produce the cells for administration to the patient.

Janssen also has a CAR T cell therapy (JNJ-4528) for multiple myeloma in development which could compete with idecabtagene vicleucel. Janssen expects to file an application for their product by the end of 2020.

For reference, the WAC for Tecartus™ (brexucabtagene autoleucel), a recently approved CAR T cell therapy for mantle cell lymphoma, is $373,000.

- Advantages: Potentially the first CAR T cell therapy for multiple myeloma, promising response rates, one-time infusion
- Disadvantages: Lack of long-term remission data, crowded marketplace, high cost, possible boxed warning and REMS program, treatment delays due to preparation needed for cells, potential future competition with Janssen's CAR T cell therapy
- Reference WAC (Tecartus): $373,000

Belumosudil (Brand Name: To be determined)

Manufacturer: Kadmon
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 1Q 2021 (***FDA is reviewing belumosudil under the Real-Time Oncology Review pilot program***)

Therapeutic use

Belumosudil is in development for the treatment of patients with chronic graft-versus-host disease (GVHD) after failure of two or more lines of systemic therapy.

- Treatment of patients with chronic GVHD after failure of two or more lines of systemic therapy

Chronic GVHD is common complication of hematopoietic stem cell transplantation (HCT). It occurs when immune cells transplanted from a donor (the graft) recognize the transplant recipient (the host) as foreign, thereby initiating an immune reaction that causes disease in the transplant recipient. Chronic GVHD describes GVHD that lasts longer than 100 days and usually persists long after transplant. The signs and symptoms include skin rash, liver dysfunction intestinal problems, dry mucosa, and lung complications. Chronic GVHD is one of the major causes of transplant-related mortality after HCT. The 5-year overall survival of chronic GVHD is 55%.

The first line treatment for chronic GVHD includes corticosteroids (eg, prednisone). An estimated 14,000 people are living with chronic GVHD and an estimated 7,000 to 10,000 require additional systemic therapy beyond steroids (eg, additional immunosuppressant therapy).

Clinical profile

Belumosudil is a selective inhibitor of Rho-associated coiled-coil kinase 2 (ROCK2), a signaling pathway that modulates inflammatory response.

- ROCK2 inhibitor
- Oral formulation
- ORR: 73% with belumosudil once daily and 74% with belumosudil twice daily
- Common AEs: Fatigue, diarrhea, nausea, edema, cough, dyspnea
- Dosing: Once or twice daily

<u>Pivotal trial data:</u>
The efficacy of belumosudil was evaluated in ROCKstar, a Phase 2, open-label trial in 132 adults and adolescents with chronic GVHD who have received at least two prior lines of systemic therapy. Patients were randomized to receive belumosudil 200 mg once daily or 200 mg twice daily. The primary endpoint was the ORR, defined as the percentage of patients who achieve a complete or partial response at any time point during the study, per the 2014 National Institutes of Health (NIH) overall response criteria.

At 6 months, the ORR was 73% with 200 mg once daily (95% CI: 60, 83; $p < 0.0001$) and 74% with 200 mg twice daily (95% CI: 62, 84; $p < 0.0001$). Responses were achieved across key patient subgroups and complete responses were observed in all organ systems. While data continue to mature, 49% of responders have maintained their response for at least 20 weeks at the time of the primary analysis. With a median treatment duration of 29 weeks, the median duration of response has not yet been reached in this ongoing study.

<u>Safety:</u>
The most common adverse events with belumosudil use were fatigue, diarrhea, nausea, edema, cough, and dyspnea.

<u>Dosing:</u>
In the pivotal trial, belumosudil was administered orally once daily or twice daily; the ORR was similar for both dosing frequencies.

Belumosudil (continued...)

Competitive environment

If approved, belumosudil would offer a novel MOA for the treatment of chronic GVHD. The frontline treatment for chronic GVHD is corticosteroid therapy but most patients require additional immunosuppressive agents, such as calcineurin inhibitors (eg, cyclosporine, tacrolimus). In 2017, the FDA approved Pharmacyclics' Imbruvica® (ibrutinib) for the treatment of chronic GVHD after failure of one or more treatments. Incyte's Jakafi® (ruxolitinib) is also approved in the steroid-refractory acute GVHD. The response rates of greater than 70% with belumosudil were promising in the refractory chronic GVHD setting as FDA guidance only requires a rate ≥ 30% for clinical significance. Based on the available information, belumosudil was relatively well tolerated with adverse events consistent with those expected to be observed in chronic GVHD patients receiving corticosteroids.

The initial indication for belumosudil is expected to be narrow as chronic GVHD requiring systemic therapy is already rare and the pivotal trial for belumosudil required failure of two or more lines of systemic therapy. While it is difficult to compare across clinical trials due to differences in patient populations, response rates appear similar to Imbruvica although 34% of patients in the pivotal trial had prior use with Imbruvica. Since the trial did not include a comparator arm, it is difficult to assess the efficacy of belumosudil vs. Imbruvica or other alternatives used for refractory chronic GVHD cases.

Belumosudil is also in early development for treatment of systemic sclerosis (SSc), a chronic immune disorder characterized by fibrosis of the skin and internal organs. SSc affects approximately 75,000 people in the U.S. Enrollment of a Phase 2 trial was delayed due to the ongoing COVID-19 pandemic but if future data is positive for SSc, that would significantly increase the market potential for belumosudil.

For reference, the WAC for Imbruvica is approximately $13,900 per 30 days.

- Advantages: Novel MOA, promising response rates, well tolerated, potential future use in systemic sclerosis
- Disadvantages: Narrow initial indication, lack of head-to-head data vs. backline treatment options for chronic GVHD
- Reference WAC (Imbruvica): ~$13,900 per 30 days

4th Quarter 2020

Extended generic pipeline forecast

OptumRx generic pipeline forecast

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
2020 Possible launch date					
CUVPOSA	glycopyrrolate	Merz	Oral solution	All	2020
PREPOPIK	citric acid/magnesium oxide/sodium picosulfate	Ferring Pharmaceuticals	Oral solution	All	2020
SUPRENZA	phentermine	Citius/Akrimax	Tablet, orally disintegrating	All	2020
PRESTALIA	perindopril/amlodipine	Symplmed	Tablet	All	2020
DORYX MPC	doxycycline hyclate	Mayne	Tablet, delayed-release	All	2020
ENTEREG	alvimopan	Merck	Capsule	All	2H-2020
OSMOPREP	sodium biphosphate/sodium phosphate	Bausch Health	Tablet	All	2H-2020
CHANTIX	varenicline	Pfizer	Tablet	All	4Q-2020
ULTRAVATE	halobetasol	Sun	Lotion	All	4Q-2020
OMNARIS	ciclesonide	Covis	Intranasal	All	4Q-2020
SYNDROS	dronabinol	Insys Therapeutics	Oral solution	All	4Q-2020
RESTASIS	cyclosporine	Allergan	Ophthalmic	All	4Q-2020
VIVLODEX	meloxicam	Iroko/iCeutica	Capsule	All	4Q-2020
BYETTA	exenatide	AstraZeneca	Subcutaneous	All	4Q-2020
DUREZOL	difluprednate	Alcon	Ophthalmic	All	4Q-2020
VASCEPA	icosapent ethyl	Amarin	Capsule	All	4Q-2020
XOLEGEL	ketoconazole	Almirall	Gel	All	11-2020
EPIDUO FORTE	adapalene/benzoyl peroxide	Galderma	Gel	All	12-2020
OFIRMEV	acetaminophen	Mallinckrodt	Intravenous	All	12-2020
ABSORICA	isotretinoin	Sun	Capsule	All	12-2020
TOVIAZ	fesoterodine	Pfizer	Tablet, extended-release	All	12-2020
DALIRESP	roflumilast	AstraZeneca	Tablet	All	12-2020
DEXILANT	dexlansoprazole	Takeda	Capsule, delayed-release	All	12-2020
VELPHORO	sucroferric oxyhydroxide	Vifor Fresenius Medical Care Renal Pharma (VFMCRP)	Tablet, chewable	All	12-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
SAPHRIS	asenapine	Allergan	Tablet, sublingual	All	12-2020
2021 Possible launch date					
BEPREVE	bepotastine	Bausch Health	Ophthalmic	All	2021
THALOMID	thalidomide	Celgene	Capsule	All	2021
AMITIZA	lubiprostone	Sucampo/Takeda	Capsule	All	01-2021
CRIXIVAN	indinavir	Merck	Capsule	All	02-2021
NORTHERA	droxidopa	H. Lundbeck	Capsule	All	02-2021
MYALEPT	metreleptin	Aegerion	Subcutaneous	All	02-2021
FORTICAL	calcitonin salmon recombinant	Upsher-Smith	Intranasal	All	02-2021
IMPAVIDO	miltefosine	Knight Therapeutics	Capsule	All	03-2021
ACTOPLUS MET XR	pioglitazone/metformin	Takeda	Tablet, extended-release	All	03-2021
NEUPRO	rotigotine	UCB	Transdermal patch	All	03-2021
POMALYST	pomalidomide	Celgene	Capsule	All	2Q-2021
LYRICA CR	pregabalin	Pfizer	Tablet, extended-release	All	04-2021
ERAXIS	anidulafungin	Pfizer	Intravenous	All	04-2021
FORTEO	teriparatide	Eli Lilly	Injection	All	04-2021
ZOMIG	zolmitriptan	Impax/Grunenthal	Intranasal	All	05-2021
PERFOROMIST	formoterol fumarate	Mylan	Inhalation	All	06-2021
INTELENCE	etravirine	Janssen	Tablet	All	06-2021
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2H-2021
NARCAN	naloxone	Emergent BioSolutions	Intranasal	All	2H-2021
LUCENTIS	ranibizumab	Roche	Intravitreal	All	2H-2021
FERAHEME	ferumoxytol	AMAG Pharmaceuticals	Intravenous	All	07-2021
RESCULA	unoprostone isopropyl	R-Tech Ueno	Ophthalmic	All	07-2021
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Tablet	All	08-2021
SUTENT	sunitinib	Pfizer	Capsule	All	08-2021
JEVTANA KIT	cabazitaxel	Sanofi	Intravenous	All	09-2021
BYSTOLIC	nebivolol	Allergan	Tablet	All	09-2021
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Capsule	All	4Q-2021
INNOPRAN XL	propranolol	Ani Pharmaceuticals	Capsule, extended-release	All	10-2021
MIRCERA	methoxy polyethylene glycol-epoetin beta	Roche/Royalty Pharma	Subcutaneous	All	11-2021

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
BROVANA	arformoterol	Sunovion	Inhalation	All	11-2021
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	Gel	All	12-2021
EPANED KIT	enalapril	Silvergate	Oral solution	All	12-2021
CAYSTON	aztreonam lysine	Gilead	Inhalation	All	12-2021
2022 Possible launch date					
PREZISTA	darunavir	Janssen	Tablet	75 mg, 150 mg, 300 mg	2022
DULERA	formoterol fumarate/mometasone furoate	Merck	Inhalation	All	2022
NATPARA	parathyroid hormone 1-84	NPS/Nycomed	Subcutaneous	All	01-2022
NPLATE	romiplostim	Amgen	Subcutaneous	All	01-2022
OXAYDO	oxycodone	Egalet	Tablet	All	01-2022
SELZENTRY	maraviroc	ViiV Healthcare	Tablet	All	02-2022
VIMPAT	lacosamide	UCB	Intravenous; tablet; oral solution	All	03-2022
ZIPSOR	diclofenac potassium	Depomed	Capsule	All	03-2022
CHOLBAM	cholic acid	Retrophin	Capsule	All	03-2022
ABRAXANE	paclitaxel	Celgene/Abraxis	Injection	All	03-2022
REVLIMID	lenalidomide	Bristol-Myers Squibb/Celgene	Capsule	All	03-2022
ARESTIN	minocycline hydrochloride	Bausch Health	Subgingival, sustained-release	All	03-2022
MAVENCLAD	cladribine	Serono	Tablet	All	03-2022
LEXISCAN	regadenoson	Astellas	Intravenous	All	04-2022
COMBIGAN	brimonidine/timolol	Allergan	Ophthalmic	All	04-2022
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	04-2022
ZOLADEX	goserelin	TerSera Therapeutics	Subcutaneous	All	04-2022
BANZEL	rufinamide	Eisai	Tablet; suspension	All	05-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
VELCADE	bortezomib	Takeda	Intravenous	All	05-2022
TARGINIQ ER	oxycodone/naloxone	Purdue	Tablet, extended-release	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Tablet	All	06-2022
VIIBRYD	vilazodone	Forest/Allergan	Tablet	All	06-2022
ELESTRIN	estradiol	Mylan	Gel	All	06-2022
QBRELIS	lisinopril	Silvergate	Oral solution	All	06-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
IRESSA	gefitinib	AstraZeneca	Tablet	All	07-2022
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	Transdermal solution	All	07-2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	07-2022
VOSEVI	sofosbuvir/velpatasvir/voxilaprevir	Gilead	Tablet	All	07-2022
VIBATIV	telavancin	Theravance	Intravenous	All	08-2022
KEVEYIS	dichlorphenamide	Strongbridge Biopharma	Tablet	All	08-2022
ORAVIG	miconazole	Midatech/R-Pharm	Tablet, buccal	All	09-2022
BIJUVA	estradiol/progesterone	TherapeuticsMD	Capsule	All	09-2022
HALFLYTELY with BISACODYL	bisacodyl / polyethylene glycol 3350, potassium chloride, sodium bicarbonate, sodium chloride	Braintree	Tablet/oral solution	All	10-2022
ORENCIA	abatacept	Bristol-Myers Squibb	Intravenous; subcutaneous	All	11-2022
XERESE	acyclovir/hydrocortisone	Bausch Health	Cream	All	11-2022
NAGLAZYME	galsulfase	BioMarin	Intravenous	All	11-2022
FOLOTYN	pralatrexate	Acrotech/Aurobindo	Intravenous	All	11-2022
GLOPERBA	colchicine	Avion Pharmaceuticals	Oral solution	All	11-2022
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	12-2022
MYRBETRIQ	mirabegron	Astellas	Tablet, extended-release	All	12-2022
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	12-2022
RAYOS	prednisone	Horizon	Tablet, delayed-release	All	12-2022
TREANDA	bendamustine	Cephalon/Teva	Intravenous	All	12-2022
ZIOPTAN	tafluprost	Akorn	Ophthalmic	All	12-2022
2023 Possible launch date					
ALPHAGAN P	brimonidine	Allergan	Ophthalmic	All	2023
KOMBIGLYZE XR	saxagliptin/metform	Astra Zeneca	Tablet, extended-release	All	1H-2023
ONGLYZA	saxagliptin	AstraZeneca	Tablet	All	1H-2023
AMZEEQ	minocycline	Foamix	Foam	All	1Q-2023
FIRVANQ KIT	vancomycin	Azurity	Oral solution	All	1Q-2023
NOXAFIL	posaconazole	Merck	Intravenous	All	01-2023
HUMIRA	adalimumab	AbbVie	Subcutaneous	All	01-2023
PROLENSA	bromfenac	Bausch Health	Ophthalmic	All	01-2023

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
APIDRA	insulin glulisine recombinant	Sanofi	Subcutaneous	All	01-2023
DUEXIS	ibuprofen/famotidine	Horizon Pharma	Tablet	All	01-2023
XYREM	sodium oxybate	Jazz	Oral solution	All	01-2023
CAMBIA	diclofenac potassium	Depomed	Oral solution	All	01-2023
TROKENDI XR	topiramate	Supernus	Capsule, extended-release	All	01-2023
DUOBRII	halobetasol propionate/tazarotene	Bausch Health	Lotion	All	01-2023
LUMIZYME	alglucosidase alfa	Genzyme	Intravenous	All	02-2023
LATUDA	lurasidone	Sunovion	Tablet	All	02-2023
GATTEX	teduglutide recombinant	Takeda	Subcutaneous	All	03-2023
AGGRASTAT	tirofiban	Medicure	Intravenous	All	03-2023
AUBAGIO	teriflunomide	Sanofi/Genzyme	Tablet	All	03-2023
DEFITELIO	defibrotide	Jazz	Intravenous	All	03-2023
PROVAYBLUE	methylene blue	Provepharm/American Regent	Intravenous	All	04-2023
KEPIVANCE	palifermin	Swedish Orphan Biovitrum	Intravenous	All	04-2023
CLINDESSE	clindamycin phosphate	Perrigo	Vaginal cream	All	04-2023
CORLANOR	ivabradine	Amgen	Tablet	All	04-2023
DALVANCE	dalbavancin	Amgen	Intravenous	All	05-2023
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Tablet	All	05-2023
EYLEA	aflibercept	Regeneron	Intraocular	All	06-2023

+ = may launch during the stated date or later

Extended brand pipeline forecast

OptumRx Brand Pipeline Forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2020 Possible launch date									
EBP-994 (Sarasar)	lonafarnib	Eiger Biopharmaceuticals	prenylation inhibitor	Progeria and progeroid laminopathies	PO	Filed NDA	11/20/2020	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Filed NDA	11/24/2020	Yes	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	11/25/2020	Yes	No
BIM-22493 (RM-493)	setmelanotide	Rhythm Pharmaceuticals	melanocortin 4 receptor (MC4R) agonist	Rare genetic disorders of obesity	SC	Filed NDA	11/27/2020	Yes	Yes
3-F8 (Hu-3F8)	naxitamab	Y-mAbs Therapeutics	GD2 antagonist	Neuroblastoma	IV	Filed BLA	11/30/2020	Yes	Yes
Tlando	testosterone	Lipocine	androgen	Hypogonadism	PO	Filed NDA	11/30/2020	No	No
Hetlioz (oral liquid)	tasmelton	Vanda	melatonin receptor 1 and 2 agonist	Smith-Magenis Syndrome	PO	Filed NDA	12/1/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder/ Pain	SC	Tentative Approval	12/1/2020	Yes	No
BCX-7353	berotralstat	BioCryst	kallikrein inhibitor	Hereditary angioedema	PO	Filed NDA	12/3/2020	Yes	Yes
ALNG-01 (ALN-G-01)	lumasiran	Alnylam	glycolate oxidase antagonist	Hyperoxaluria	SC	Filed NDA	12/3/2020	Yes	Yes
MAGH-22	margetuximab	MacroGenics	HER2 oncoprotein antagonist	Breast cancer	IV	Filed BLA	12/18/2020	Yes	No
FG-4592	roxadustat	FibroGen/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase inhibitor	Anemia	PO	Filed NDA	12/20/2020	Yes	No
TAK-385	relugolix	Myovant Sciences	gonadotropin-releasing hormone receptor antagonist	Prostate cancer	PO	Filed NDA	12/20/2020	Yes	No
MK-4618 (KRP-114V, RVT-901)	vibegron	Urovant Sciences	selective beta 3 adrenergic receptor agonist	Overactive bladder	PO	Filed NDA	12/26/2020	No	No
LY-03005	ansofaxine	Luye Pharma	serotonin-norepinephrine-dopamine triple reuptake inhibitor	Major depressive disorder	PO	Filed NDA	12/26/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ALN-PCSsc (PCSK9si)	inclisiran	Novartis	RNA interfering therapeutic targeting proprotein convertase subtilisin–kexin type 9 (PCSK9)	Hyperlipidemia	SC	Filed NDA	12/2020	Yes	Yes
SPI-2012	eflapegrastim	Spectrum	granulocyte colony-stimulating factor (GCSF)	Chemotherapy-induced neutropenia	SC	Filed BLA	4Q2020	Yes	No
TSR-042	dostarlimab	GlaxoSmithKline	PD-1 checkpoint inhibitor	Endometrial cancer	IV	Filed BLA	4Q2020	Yes	No
Leukotac	inolimomab	ElsaLys Biotech	IL-2 monoclonal antibody	Graft vs. host disease	IM	Filed BLA	4Q2020	Yes	Yes
Ontinua ER	arbaclofen extended-release	Osmotica	muscle relaxant	Multiple sclerosis	PO	Filed NDA	12/29/2020	Yes	No
KX-01 (KX2-391)	tirbanibulin	Athenex	Src kinase and tubulin inhibitor	Actinic keratosis	TOP	Filed NDA	12/30/2020	No	No
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	Filed NDA	12/30/2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2021 Possible launch date									
BAY-1021189 (MK-1242)	vericiguat	Merck/ Bayer	guanylate cyclase stimulator	Heart failure	PO	Filed NDA	1/20/2021	Yes	No
Luveniq	voclosporin	Aurinia Pharmaceuticals	calcineurin inhibitor	Lupus nephritis	PO	Filed NDA	1/22/2021	Yes	No
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement therapy	Fabry disease	IV	Filed BLA	1/27/2021	Yes	No
mAb114	ansuvimab	Ridgeback Therapeutics	Monoclonal antibody	Ebola	IM	Filed BLA	1/28/2021	No	Yes
REGN-1500	evinacumab	Regeneron	angiopoietin-like 3 (ANGPTL3) antagonist	Homozygous familial hypercholesterolemia	IV	Filed BLA	2/11/2021	Yes	Yes
TGR-1202	umbralisib	TG Therapeutics	phosphoinositide-3 kinase (PI3K) delta inhibitor	Marginal zone lymphoma/ follicular lymphoma	PO	Filed NDA	2/15/2021	Yes	Yes
GZ-381 (G1-T28)	trilaciclib	G1 Therapeutics/ Boehringer Ingelheim	Cyclin dependent kinase inhibitor	Small cell lung cancer	IV	Filed NDA	2/15/2021	Yes	No
SRP-4045	casimersen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	Filed BLA	2/25/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Neutrolin	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	Filed NDA	2/28/2021	No	No
HM30181A/paclitaxel	paclitaxel and encequidar	Athenex	P-glycoprotein pump inhibitor/ taxane	Breast cancer	PO	Filed NDA	2/28/2021	Yes	No
Zydena	udenafil	Mezzion Pharma	phosphodiesterase type 5 (PDE5) inhibitor	Congenital single ventricle heart disease	PO	Filed NDA	2/28/2021	No	Yes
CPP-1X/ sulindac (DFMO)	eflornithine/ sulindac	Cancer Prevention Pharma	ornithine decarboxylase inhibitor/ non-steroidal anti-inflammatory drug (NSAID)	Familial adenomatous polyposis	PO	Filed NDA	2/28/2021	Yes	Yes
Ygalo (Melflufen)	melphalan-flufenamide	Oncopeptides AB	alkylating agent/ DNA synthesis inhibitor	Multiple myeloma	IV	Filed NDA	2/28/2021	No	Yes
KP-415	D-threo-methylphenidate controlled-release	KemPharm	CNS stimulant	Attention deficit hyperactivity disorder	PO	Filed NDA	3/2/2021	No	No
BIIB-037	aducanumab	Biogen	amyloid beta-protein inhibitor	Alzheimer's disease	IV	Filed BLA	3/7/2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease type C	PO	Filed NDA	3/17/2021	Yes	Yes
RG-3477 (ACT-128800)	ponesimod	Johnson & Johnson	sphingosine 1 phosphate receptor agonist	Multiple sclerosis	PO	Filed NDA	3/18/2021	Yes	No
bb-2121	idecabtagene vicluecel	Bristol-Myers Squibb/ bluebird Bio	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	Filed BLA	3/27/2021	Yes	Yes
ZP-4207	dasiglucagon	Zealand Pharma	glucagon analog	Diabetes mellitus	SC	Filed NDA	3/27/2021	No	Yes
TMC-278-LA	cabotegravir (long-acting)/ rilpivirine (long-acting)	ViiV Healthcare	HIV integrase inhibitor/ non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV	IM	Filed NDA	1Q2021	Yes	No
S-265744 (S/GSK-1265744)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	HIV	PO	Filed NDA	1Q2021	Yes	No
KD-025	belumosudil	Kadmon	Rho-associated coiled-coil kinase 2 inhibitor	Graft vs. Host disease	PO	Filed NDA	1Q2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Hydexor	promethazine/ hydrocodone/ acetaminophen	Charleston Laboratories	anti-emetic/ opioid/ analgesic	Nausea/ Vomiting/ Pain	PO	Filed NDA	1Q2021	No	No
EMD-1214063	tepotinib	Merck	c-Met receptor tyrosine kinase inhibitor	Non-small cell lung cancer	PO	Filed NDA	1Q2021	Yes	No
Tivopath	tivozanib	AVEO Oncology	VEGF inhibitor	Renal cell cancer	PO	Filed NDA	3/31/2021	Yes	No
Ropeg	ropeginterferon alfa-2b	PharmaEssentia	interferon	Polycythemia vera	SC	Filed BLA	3/2021 - 4/2021	Yes	Yes
cyclic pyranopterin monophosphate (ALXN-1101)	fosdenopterin	BridgeBio Pharma/ Origin Biosciences	molybdenum cofactor stimulant	Molybdenum cofactor deficiency	IV	Filed NDA	4/11/2021	Yes	Yes
Estelle	estetrol/ drospirenone	Mayne Pharma/ Mithra Pharmaceuticals	estrogen receptor agonist	Pregnancy prevention	PO	Filed NDA	4/16/2021	No	No
S5G4T-1 (DER-45-EV)	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	Filed NDA	4/26/2021	No	No
PF-04965842	abrocitinib	Pfizer	janus kinase 1 (JAK-1) inhibitor	Atopic dermatitis	PO	Filed NDA	4/2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
APL-2	pegcetacoplan	Apellis Pharmaceuticals	complement C3 inhibitor	Paroxysmal nocturnal hemoglobinuria	SC	Filed NDA	5/14/2021	Yes	Yes
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	Filed NDA	5/27/2021	No	Yes
FP-001 (LMIS)	leuprolide mesylate	Foresee	gonadotropin-releasing hormone (GnRH) analog	Prostate cancer	SC	Filed NDA	5/27/2021	Yes	No
ET-104	zonisamide	Eton	anticonvulsant	Seizures	PO	Filed NDA	5/29/2021	No	No
TAK-721 (SHP-621)	budesonide	Takeda	corticosteroid	Eosinophilic esophagitis	PO	Filed NDA	5/30/2021	Yes	Yes
relugolix/ estradiol/ norethindrone acetate	relugolix/ estradiol/ norethindrone acetate	Myovant Sciences	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids	PO	Filed NDA	6/1/2021	No	No
Ryplazim	human plasminogen	Liminal BioSciences	plasminogen	Plasminogen deficiency	IV	Filed BLA	6/5/2021	Yes	Yes
StrataGraft Skin Tissue	StrataGraft Skin Tissue	Mallinckrodt	autologous skin tissue	Burn injury	TOP	Filed BLA	6/8/2021	Yes	Yes
SCY-078 (MK-3118)	ibrexafungerp	Scynexis	glucan synthase inhibitors	Fungal infections	PO	Filed NDA	6/14/2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ACP-001 (TransCon Growth Hormone)	lonapegsomatropin	Ascendis Pharma	growth hormone prodrug	Short stature/ growth hormone deficiency	SC	Filed BLA	6/25/2021	Yes	Yes
Verkazia	cyclosporine	Santen Pharmaceutical	immunosuppressant	Vernal keratoconjunctivitis	OPH	Filed NDA	6/26/2021	No	Yes
NexoBrid	bromelain	Vericel	peptide hydrolase replacement agent	Burns/ Skin injury	TOP	Filed BLA	6/29/2021	No	Yes
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	Filed BLA	6/2021	Yes	No
tanezumab	tanezumab	Pfizer/ Eli Lilly	nerve growth factor (NGF) inhibitor	Osteoarthritis	SC	Filed BLA	2Q2021	Yes	No
CAT-354	tralokinumab	Leo Pharma	interleukin-13 (IL-13) inhibitor	Atopic dermatitis	SC	Filed BLA	2Q2021	Yes	No
JCAR-017	lisocabtagene maraleucel	Bristol-Myers Squibb/ Celgene	chimeric antigen receptor (CAR) T cell therapy	Diffuse large B-cell lymphoma	IV	Filed BLA	1H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	CRL	1H2021	No	No
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	CRL	Mid-2021	No	Yes
ISIS 304801 (ISIS-APOCIIIRx)	volanesorsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	CRL	Mid-2021	Yes	Yes
BGJ-398	infigratinib	BridgeBio	FGFR inhibitor	Biliary tract cancer	PO	InTrial	Mid-2021	Yes	Yes
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV/SC	InTrial	Mid-2021	Yes	Yes
AGEN-2034	balstilimab	Agenus	PD-1 antagonist	Cervical cancer	IV	InTrial	Mid-2021	Yes	No
Vicinium (VB-4-845)	oportuzumab monatox	Sesen Bio	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	InTrial	Mid-2021	Yes	No
GZ-402666 (NeoGAA)	avalglucosidase alfa	Sanofi	enzyme replacement therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
JNJ-4528 (LCAR-B38M)	ciltacabtagene autoleucel	Janssen	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	InTrial	Mid-2021	Yes	Yes
HTX-011	bupivacaine/ meloxicam	Heron Therapeutics	anesthetic/ Nonsteroidal Anti-inflammatory Drug (NSAID)	Pain	Instillation	CRL	Mid-2021	No	No
AB-103	reltecimod	Atox Bio	CD-28 co-stimulatory receptor modulator	Bacterial infections	IV	InTrial	Mid-2021	Yes	Yes
CLS-1001	triamcinolone acetonide	Clearside	corticosteroid	Macular edema	Intraocular/ subretinal	CRL	Mid-2021	Yes	No
PRV-031	teplizumab	Provention Bio/ MacroGenics	CD3 antigen inhibitor	Diabetes mellitus	IV	Filed BLA	7/2/2021	Yes	Yes
CMX-001	brincidofovir	Chimerix/ SymBio Pharmaceuticals	DNA-directed DNA polymerase inhibitor	Smallpox	PO	Filed NDA	7/6/2021	No	Yes
CCX-168	avacopan	ChemoCentryx	C5a receptor (C5aR) antagonist	Vasculitis	PO	Filed NDA	7/7/2021	Yes	Yes
Uptravi (IV)	selexipag	Janssen	non-prostanoid prostacyclin agonist	Pulmonary arterial hypertension	IV	Filed NDA	7/30/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Plaque psoriasis	IV	Filed BLA	7/2021	Yes	No
ET-101	topiramate	Eton	undisclosed	Seizure disorders	PO	Filed NDA	8/6/2021	No	No
BMN-111	vosoritide	BioMarin	C-type natriuretic peptide (CNP) analog	Achondroplasia	SC	Filed NDA	8/20/2021	Yes	Yes
paliperidone palmitate (6-month)	paliperidone palmitate	Johnson & Johnson	atypical antipsychotic	Schizophrenia	IM	Filed NDA	9/2/2021	Yes	No
INP-104	POD-dihydroergotamine mesylate (POD-DHE)	Impel NeuroPharma	ergot derivative	Acute migraines	Intranasal	Filed NDA	9/9/2021	No	No
ADCT-402	loncastuximab tesirine	ADC Therapeutics	antibody drug conjugate	Diffuse large B-cell lymphoma	IV	Filed BLA	9/21/2021	Yes	Yes
TAK-788	mobocertinib	Takeda	tyrosine kinase inhibitor	Non-small cell lung cancer	PO	InTrial	3Q2021	No	Yes
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	InTrial	3Q2021	Yes	Yes
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	3Q2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ABI-009	sirolimus and albumin	Aadi Bioscience	mTOR kinase inhibitor	Epithelioid cell caricnoma	IV	InTrial	3Q2021	Yes	Yes
BAY 94-8862	finerenone	Bayer	mineralocorticoid receptor antagonist	Diabetic nephropathy	PO	Filed NDA	11/9/2021	No	No
Purified Cortrophin Gel	corticotropin	ANI Pharmaceuticals	adrenocorticotropic hormone (ACTH)	Multiple sclerosis/ rheumatoid arthritis/ systemic lupus erythematosus/ ulcerative colitis	IV	InTrial	4Q2021	Yes	No
APR-246	eprenetapopt	Aprea Therapeutics	p53 tumor suppressor protein stimulator	Myelodysplastic syndrome	IV	InTrial	4Q2021	Yes	Yes
V-114	pneumococcal conjugate vaccine	Merck	vaccine	Bacterial infection	IM	InTrial	4Q2021	Yes	No
INC-424	ruxolitinib	Incyte	janus kinase (JAK) inhibitor	Atopic dermatitis	TOP	InTrial	4Q2021	Yes	No
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	InTrial	4Q2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate (NMDA) antagonist/ antidepressant	Treatment-resistant depression	PO	InTrial	4Q2021	No	No
MOD-401	somatrogon	Opko	enzyme replacement	Growth hormone deficiency	SC	InTrial	4Q2021	Yes	Yes
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	InTrial	4Q2021	No	No
OS-01 nasal spray	OC-01	Oyster Point Pharma	nicotinic acetylcholine receptor (nAChR) agonist	Dry eye disease	Intranasal	InTrial	4Q2021	No	No
CR-845	difelikefalin	Cara Therapeutics	opioid receptor agonist	Pruritus	IV/PO	InTrial	4Q2021	No	No
BIVV-009	sutimlimab	Sanofi	complement C1s subcomponent inhibitor	Cold agglutinin disease	IV	CRL	2H2021	Yes	Yes
TAK-609	idursulfase-IT	Takeda	enzyme replacement	Hunter syndrome	Intrathecal	InTrial	2H2021	Yes	Yes
PL-56	budesonide	Calliditas	corticosteroid	Nephropathy	PO	InTrial	2H2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RTA-402	bardoxolone methyl	Reata Pharmaceuticals/ AbbVie	Nrf2 activator	Alport syndrome	PO	InTrial	2H2021	Yes	Yes
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist (RAR-gamma)	Fibrodysplasia ossificans progressiva (FOP)	PO	InTrial	2H2021	Yes	Yes
ARGX-113	efgartigimod	Argen NV	Fc antagonist	Myasthenia gravis	IV/SC	InTrial	2H2021	Yes	Yes
odevixibat	odevixibat	Albierio	ileal bile acid transporter inhibitor	Progressive familial intrahepatic cholestasis	PO	InTrial	2H2021	Yes	Yes
HMPL-012	surufatinib	Hutchison China MediTech	angio-immunokinase inhibitor	Neuroendocrine tumors	PO	InTrial	2H2021	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Intrathecal	InTrial	2H2021	Yes	Yes
SPN-812	viloxazine	Supernus Pharmaceuticals	selective norepinephrine reuptake inhibitor	Attention deficit hyperactivity disorder	PO	CRL	2H2021	No	No
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	InTrial	2H2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MTP-131 (SS-31)	elamipretide	Stealth Biotherapeutics	mitochondrial permeability transition pore inhibitor	Barth syndrome	IV/PO/SC	InTrial	2H2021	Yes	Yes
AmnioFix	dehydrated human amnion/chorion membrane (dHACM)	MiMedx	amniotic tissue membrane	Plantar fasciitis/ achilles tendonitis	INJ	InTrial	2H2021	Yes	No
ublituximab (LFB-R603, TG20, TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia/ multiple sclerosis	IV	InTrial	2H2021	Yes	Yes
SGX-301	synthetic hypericin	Access Pharmaceuticals	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	2H2021	Yes	Yes
TG-1303	ublituximab/ TGR-1202	TG Therapeutics	CD-20 monoclonal antibody/ phosphoinositide-3 kinase (PI3K) delta inhibitor	Chronic lymphocytic leukemia/ Non-Hodgkin lymphoma	IV/PO	InTrial	2H2021	Yes	Yes
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Bacterial infection	IV/PO	InTrial	2H2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
dovitinib	dovitinib	Oncology Venture	fibroblast growth factor receptor 3 (FGFR3) inhibitor	Renal cell carcinoma	PO	InTrial	2H2021	Yes	No
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent (tVDA)	Neutropenia/ non-small cell lung cancer	IV	InTrial	2H2021	Yes	No
Instiladrin	nadofaragene firadenovec	Ferring Pharmaceuticals/ Blackstone Life Sciences	gene therapy	Bladder cancer	Intravesical	CRL	2H2021	Yes	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	2H2021	Yes	Yes
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	InTrial	2H2021	Yes	No
PDR-001	spartalizumab	Novartis	PD-1 checkpoint inhibitor	Melanoma	IV	InTrial	2H2021	Yes	No
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	InTrial	2H2021	No	No
ABT-888	veliparib	AbbVie	poly (ADP-ribose) polymerase (PARP) inhibitor	Ovarian cancer; breast cancer	PO	InTrial	2H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer; breast cancer	PO	InTrial	2H2021	Yes	No
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	2H2021	Yes	No
SYD-985	[vic-] trastuzumab duocarmazine	Synthon	HER2-targeting antibody-drug conjugate	Breast cancer	IV	InTrial	2H2021	Yes	No
Taclantis	paclitaxel injection concentrate for suspension	Sun Pharma Advanced Research Company (SPARC)	taxane	Breast cancer; lung cancer; pancreatic cancer	IV	CRL	2H2021	No	No
SHP-620	maribavir	Shire	benzimidazole	Cytomegalovirus	PO	InTrial	2H2021	No	Yes
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis/ Crohn's disease	SC	CRL	2H2021	Yes	No
JZP-458 (PF-743)	recombinant crisantaspase	Jazz Pharmaceutics/ Pfenex	asparaginase	Acute lymphoblastic leukemia	IM/IV	InTrial	2H2021	Yes	No
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	2H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
BBI-608	napabucasin	Sumitomo Dainippon	stem cell inhibitor	Colorectal cancer	PO	InTrial	2H2021	Yes	No
AGEN-1884	zalifrelimab	Agenus	immune checkpoint modulator (CPM) antibody	Cervical cancer	IV	InTrial	2H2021	Yes	No
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	2H2021	Yes	No
ZYN-002	ZYN-002	Zynerba	cannabinoid product	Fragile X syndrome	TOP	InTrial	2H2021	Yes	Yes
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	CRL	2021	Yes	No
ET-105	lamotrigine	Eton	anticonvulsant	Epilepsy	PO	CRL	2021	No	No
Zimhi	naloxone	Adamis	opioid antagonist	Opioid overdose	IM	CRL	Late 2021	No	No
FT-2102	olutasidenib	Forma Therapeutics	dehydrogenase 1 (IDH1) inhibitor	Acute myeloid leukemia	PO	InTrial	Late 2021	Yes	Yes
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 (IGF-1) derivative	Rett syndrome	IV/PO	InTrial	Late 2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
JNJ-6372	amivantamab	Johnson & Johnson	EGFR and cMET antibody	Non-small cell lung cancer	IV	InTrial	Late 2021	Yes	No
PDS-1.0	ranibizumab	Roche/ Genentech	Anti-VEGF (vascular endothelial growth factor)	Wet age-related macular degeneration	Intravitreal implant	InTrial	Late 2021	Yes	No
IDP-124	pimecrolimus	Bausch Health	calcineurin Inhibitor	Atopic dermatitis	TOP	InTrial	Late 2021	No	No
ACT-541468	daridorexant	Idorsia Pharmaceuticals	orexin receptor antagonist	Insomnia	PO	InTrial	Late 2021	No	No
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	undisclosed	Distal rental tubular acidosis	PO	InTrial	Late 2021	Yes	No
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	InTrial	Late 2021	No	No
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV	SC	InTrial	Late 2021	Yes	Yes
IDP-120	tretinoin/ benzoyl peroxide	Bausch	retinoid	Acne	TOP	InTrial	Late 2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	Late 2021	Yes	Yes
MYK-461 (SAR-439152)	mavacamten	MyoKardia	mysoin inhibitor	Cardiomyopathy	PO	InTrial	Late 2021	Yes	Yes
Trevyent	treprostinil	United Therapeutics	prostacyclin analog	Pulmonary arterial hypertension	SC	CRL	Late 2021	Yes	Yes
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	Late 2021	No	No
AGIL-AADC	AGIL-AADC	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	Late 2021	Yes	Yes
TadFin	tadalafil and finasteride	Veru	phosphodiesterase type 5 inhibitor /5-alpha-reductase inhibitor	Benign prostatic hyperplasia	PO	InTrial	Late 2021	No	No
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myeloid leukemia	PO	InTrial	Late 2021	Yes	Yes
AKB-6548	vadadustat	Akebia Therapeutics/ Vifor Pharma/ Otsuka	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	InTrial	Late 2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AT-GAA	recombinant human acid alpha-glucosidase + AT2220	Amicus	enzyme therapy	Pompe disease	IV	InTrial	Late 2021	Yes	Yes
pacritinib	pacritinib	CTI BioPharma	janus associated kinase-2 (JAK2) inhibitor	Myelofibrosis	PO	InTrial	Late 2021	Yes	Yes
SHP-625 (LUM-001)	maralixibat	Mirum Pharmaceuticals	apical sodium-dependent bile acid transporter (ABST) inhibitor	Alagille syndrome	PO	InTrial	Late 2021	Yes	Yes
2022 Possible launch date									
MK-8031	atogepant	AbbVie	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine prophylaxis	PO	InTrial	1Q2022	No	No
AT-007	AT-007	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	undisclosed	InTrial	1Q2022	Yes	Yes
S-265744 LAP (S/GSK-1265744 LAP; GSK-744 LA)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	Pre-exposure prophylaxis HIV	IM	InTrial	1Q2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
SPR-994	tebipenem	Spero Therapeutics	carbapenem	Urinary tract infections	PO	InTrial	1Q2022	No	No
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	InTrial	1Q2022	Yes	Yes
Filsuvez (AP-101)	episalvan	Amryt Pharma	triterpene	Epidermolysis bullosa	TOP	Not Approved	1Q2022	No	Yes
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	InTrial	2Q2022	No	No
CERC-801	CERC-801	Cerecor	D-galactose	Phosphoglucomutase 1 (PGM1) deficiency	PO	InTrial	1H2022	Yes	Yes
Zynteglo (LentiGlobin)	lentiviral beta-globin gene transfer	Bluebird Bio	gene therapy	Beta-thalassemia	IV	InTrial	1H2022	Yes	Yes
DARE-BV1	clindamycin	Daré Bioscience	lincosamide	Bacterial vaginosis	Intravaginal	InTrial	1H2022	No	No
VT-1161	oteseconazole	Mycovia Pharmaceuticals	lanosterol demethylase (CYP51) inhibitor	Fungal infections	PO	InTrial	1H2022	No	No
ACER-001	sodium phenylbutyrate	Acer Therapeutics	BCKDC kinase inhibitor	Urea cycle disorders	PO	InTrial	1H2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MLN-4924 (TAK-92)	pevonedistat	Ligand	Nedd 8 Activating Enzyme (NAE) antagonist	Myelodysplastic syndrome	IV	InTrial	1H2022	Yes	No
AMG-157 (MEDI-9929)	tezepelumab	AstraZeneca/ Amgen	thymic stromal lymphopoietin antagonist	Asthma	IV/SC	InTrial	1H2022	Yes	No
OTL-200 (GSK-2696274)	OTL-200 (GSK-2696274)	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	1H2022	Yes	Yes
Estybon	rigosertib (ON 01910.Na)	Onconova	non-ATP competitive kinase inhibitor	Myelodysplastic syndrome	IV	InTrial	1H2022	Yes	Yes
COR-003	levoketoconazole	Strongbridge Biopharma	azole antifungal	Cushing's syndrome	PO	InTrial	1H2022	No	Yes
Sativex	nabiximols	GW Pharmaceuticals/ Otsuka	cannabinoid product	Spasticity	PO	InTrial	1H2022	No	No
177Lu-PSMA-617	Lutetium	Novartis	Radiopharmaceutical	Prostate cancer	IV	InTrial	1H2022	Yes	No
OBE-2109 (KLH-2109)	linzagolix	ObsEva	gonadotropin-releasing hormone (GnRH) antagonist	Uterine fibroids	PO	InTrial	1H2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Adrenomyeloneuropathy	IV	InTrial	1H2022	Yes	Yes
REGN-2477	garetosmab	Regeneron	Activin A antibody	Fibrodysplasia ossificans progressiva	IV/SC	InTrial	Mid-2022	Yes	Yes
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	InTrial	Mid-2022	Yes	Yes
CCD-1042	ganaxolone	Marinus Pharmaceuticals	allopregnanolone analog	Seizures	PO	InTrial	Mid-2022	No	Yes
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	Mid-2022	Yes	Yes
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	InTrial	Mid-2022	Yes	Yes
MRTX-849	adagrasib	Mirati Therapeutics	KRAS inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2022	Yes	No
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	Mid-2022	Yes	Yes
OTL-103 (GSK-2696275)	OTL-103 (GSK-2696275)	Orchard Therapeutics	gene therapy	Wiskott-Aldrich syndrome	IV	InTrial	Mid-2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
M-7824	bintrafusp alfa	GlaxoSmithKline	PD-L1 / TGF-beta immunoinhibition	Biliary tract cancer	IV	InTrial	Mid-2022	Yes	Yes
DJ-927	tesetaxel	Odonate Therapeutics	Microtubules (tubulin) inhibitor	Breast cancer	PO	InTrial	Mid-2022	No	No
DCR-PHXC	nedosiran	Dicerna/ Alnylam	glycolate oxidase antagonist	hyperoxaluria	SC	InTrial	Mid-2022	Yes	Yes
MIN-102	hydroxypioglitazone	Minoryx Therapeutics	PPAR gamma agonist	Adrenomyeloneuropathy	Undisclosed	InTrial	Mid-2022	Yes	Yes
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	CRL	Mid-2022	Yes	Yes
RG-7828	mosunetuzumab	Roche	anti-CD20/CD3 monoclonal antibody	Follicular lymphoma	IV/SC	InTrial	Mid-2022	Yes	Yes
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	InTrial	Mid-2022	Yes	Yes
PT-027	budesonide/ albuterol	AstraZeneca	Glucocorticoid/beta agonist	Asthma	Inh	InTrial	Mid-2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AMT-061	etranacogene dezaparvovec	CSL Behring/ uniQure	gene therapy	Hemophilia B	IV	InTrial	Mid-2022	Yes	Yes
GZ-402665	olipudase alfa	Sanofi	sphingomyelinase	Acid sphingomyelinase deficiency	IV	InTrial	Mid-2022	Yes	Yes
WTX-101	bis-choline tetrathiomolybdate (TTM)	Alexion	chelating agent	Wilson's disease	PO	InTrial	Mid-2022	Yes	Yes
AG-348	mitapivat	Agios	pyruvate kinase-R (PKR) activator	Pyruvate kinase deficiency	PO	InTrial	Mid-2022	Yes	Yes
BHV-3500	vazegepant	Biohaven	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine	Intranasal	InTrial	4Q2022	No	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	2H2022	No	No
RG-7716 (RO-6867461)	faricimab	Roche/ Chugai	bispecific VEGF-A/ angiopoietin-2 antagonist	Diabetic macular edema; age-related macular degeneration	Intravitreal	InTrial	2H2022	Yes	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor (NK-1R) antagonist	Motion sickness	PO	InTrial	2H2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PDP-716	brimonidine	Sun Pharma Advanced Research Company (SPARC)	alpha-2 agonist	Glaucoma	OP	InTrial	2H2022	No	No
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	InTrial	2H2022	Yes	No
Oxabact	oxalobacter	OxThera	probiotic	Hyperoxaluria	PO	InTrial	2H2022	No	Yes
GLPG-0634	filgotinib	Gilead/ Galapagos	janus associated kinase-1 (JAK) inhibitor	Rheumatoid arthritis	PO	CRL	2H2022	Yes	No
QGE-031	ligelizumab	Novartis	Anti-IgE antibody	Urticaria	SC	InTrial	2H2022	Yes	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	2H2022	Yes	Yes
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	2022	Yes	Yes
FCX-007 (GM-HDF-COL7, INXN-3002)	FCX-007 (GM-HDF-COL7, INXN-3002)	Castle Creek Pharmaceutical	gene-modified autologous fibroblast	Epidermolysis Bullosa	Intradermal	InTrial	2022	Yes	Yes
HY-01	minocycline	Hovione	tetracycline	Rosacea	TOP	InTrial	2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor (FXR) agonist	Nonalcoholic steatohepatitis	PO	CRL	2022	Yes	No
scCeftriaxone	ceftriaxone	scPharmaceuticals	penicillin binding protein inhibitor	Bacterial infections	SC	InTrial	2022	No	No
glatiramer acetate depot	glatiramer acetate long-acting	Mylan	immunosuppressant	Multiple sclerosis	IM	InTrial	2022	Yes	No
KN-046	KN-046	Alphamab Oncology	PD-L1/CTLA-4 bispecific monoclonal antibody	Thymic cancer	IV	InTrial	2022	Yes	Yes
MBG-453	MBG-453	Novartis	anti-TIM-3	Myelodysplastic syndrome	IV	InTrial	2022	Yes	No
GSK-2894512 (WBI-1001)	tapinarof	Dermavant Sciences	therapeutic aryl hydrocarbon receptor modulating agent (TAMA)	Plaque psoriasis	TOP	InTrial	2022	Yes	No
REGN-475 (SAR-164877)	fasinumab	Regeneron/ Sanofi-Aventis/ Teva	selective anti-nerve growth factor (NGF) monoclonal antibody	Osteoarthritis	IV/SC	InTrial	2022	Yes	No
DBV-712 (Viaskin Peanut)	DBV-712	DBV Technologies	Immunotherapy	Peanut allergy	TOP	CRL	2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CNTX-4975	CNTX-4975	Centrexion Therapeutics	TRPV1 agonist	Osteoarthritis	Intra-articular	InTrial	2022	Yes	No
KB-103	beremagene geperpavec	Krystal Biotech	Gene therapy	Epidermolysis bullosa	Topical	InTrial	2022	Yes	Yes
VGX-3100	VGX-3100	Inovio	vaccine	Cervical cancer/dysplasia	IM	InTrial	2022	Yes	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	InTrial	2022	Yes	No
POL-6326	balixafortide	Polyphor	chemokine antagonist	Breast cancer	IV	InTrial	2022	Yes	No
iDose travoprost	travoprost	Glaukos Corporation	prostaglandin analog	Glaucoma/ Ocular hypertension	Intraocular	InTrial	2022	No	No
CM-AT	CM-AT	Curemark	protein absorption enhancer	Autism	PO	InTrial	2022	Yes	No
OTL-101	ADA-transduced autologous stem cell therapy	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	Undisclosed	InTrial	2022	Yes	Yes
CERC-802	CERC-802	Cerecor	D-mannose	Mannose-phosphate isomerase deficiency	PO	InTrial	2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RGN-259 (GBT-201; RGN-352)	timbetasin	RegeneRx	actin regulating peptide	Dry eyes	OP	InTrial	2022	No	Yes
pentoxifylline	pentoxifylline	Eton	phosphodiesterase inhibitor	Peyronie's disease	PO	InTrial	2022	No	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson’s disease	SC infusion	InTrial	2022	Yes	No
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor/ Japan Tobacco/ Roche	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	Late 2022	Yes	No
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eyes	OP	InTrial	Late 2022	No	No
NuThrax	anthrax vaccine adsorbed/ CPG-7909	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	InTrial	Late 2022	Yes	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	Late 2022	No	No
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Late 2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	Late 2022	Yes	No
ARQ-151	roflumilast	Arcutis Biotherapeutics	Phosphodiesterase-4 (PDE-4) inhibitor	Plaque psoriasis	TOP	InTrial	Late 2022	Yes	No

IM = intramuscular, INH = inhalation, INJ = injection, IUD = intrauterine device, IV = intravenous, OP = ophthalmic, PO = oral, SC = subcutaneous, TOP = topical, VG = vaginal

Key pending indication forecast

OptumRx Key Pending Indication Forecast

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Xolair	omalizumab	Novartis	IgE antagonist	Nasal polyps	Treatment of adults with chronic rhinosinusitis with nasal polyps (CRSwNP) who have not adequately responded to intranasal corticosteroids	SC	9/30/2020
Trelegy Ellipta	fluticasone furoate/ umeclidinium/ vilanterol	GlaxoSmithKline	inhaled corticosteroid (ICS)/ long-acting muscarinic agent (LAMA)/ long-acting beta agonist (LABA)	Chronic obstructive pulmonary disease	Reduction in all-cause mortality in patients with chronic obstructive pulmonary disease (COPD)	INH	10/1/2020
Linzess	linaclotide	Allergan/ Ironwood Pharmaceuticals	guanylate cyclase C receptor agonist	Abdominal symptoms	Treatment of abdominal symptoms	PO	10/31/2020
Xofluza	baloxavir	Genentech/ Shionogi	polymerase acidic (PA) endonuclease inhibitor	Influenza	Post-exposure prophylaxis of influenza in people one year of age and older	PO	11/23/2020
Xofluza	baloxavir	Genentech/ Shionogi	polymerase acidic (PA) endonuclease inhibitor	Influenza	Treatment of acute uncomplicated influenza in otherwise healthy children aged one to less than 12 years of age who have been symptomatic for no more than 48 hours	PO	11/23/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Hetlioz	tasimelteon	Vanda	melatonin receptor 1 and 2 agonist	Smith-Magenis Syndrome (SMS)	Treatment of adults with Smith-Magenis Syndrome (SMS)	PO	12/1/2020
Xeomin	incobotulinumtoxinA	Merz Pharmaceuticals	acetylcholine release inhibitor and neuromuscular blocking agent	Chronic sialorrhea	Treatment of chronic sialorrhea in pediatric patients	IM	12/1/2020
Imfinzi	durvalumab	AstraZeneca	anti-PD-L1 antibody	Non-small cell lung cancer / bladder cancer	Fixed dose (1,500 mg) given every 4 weeks for stage 3 non-small cell lung cancer and bladder cancer	IV	12/1/2020
Symdeko	tezacaftor/ ivacaftor; ivacaftor	Vertex	CFTR corrector/ CFTR potentiator	Cystic fibrosis	Treatment of patients with cystic fibrosis who have rare CFTR mutations. And will allow certain people who are eligible for Kalydeco to become eligible for Symdeko or Trikafta and certain people who are eligible for Symdeko to become eligible for Trikafta	PO	12/30/2020
Trikafta	elexacaftor/tezacaftor/ivacaftor; ivacaftor	Vertex	cystic fibrosis transmembrane conductance regulator (CFTR) modulators	Cystic fibrosis	Treatment of patients with cystic fibrosis who have rare CFTR mutations. And will allow certain people who are eligible for Kalydeco to become eligible for Symdeko or Trikafta and certain people who are eligible for Symdeko to become eligible for Trikafta	PO	12/30/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Kalydeco	ivacaftor	Vertex	CFTR activator	Cystic fibrosis	Treatment of patients with cystic fibrosis who have rare CFTR mutations. And will allow certain people who are eligible for Kalydeco to become eligible for Symdeko or Trikafta	PO	12/30/2020
Nplate	romiplostim	Amgen	thrombopoietin receptor agonist	Acute radiation syndrome	Treatment of hematopoietic syndrome of acute radiation syndrome	SC	1/28/2021
Xalkori	crizotinib	Pfizer	anaplastic lymphoma kinase (ALK) inhibitor	Anaplastic large cell lymphoma (ALCL)	Treatment of pediatric patients with relapsed or refractory systemic anaplastic large cell lymphoma (ALCL) that is anaplastic lymphoma kinase (ALK)-positive	PO	1/31/2021
Gocovri	amantadine extended-release	Adamas	NMDA receptor antagonist	Parkinson's disease	Treatment for OFF episodes in Parkinson's disease (PD) patients receiving levodopa-based therapy	PO	2/1/2021
Xolair	omalizumab	Genentech	IgE antagonist	Self-administration	Self-administration of a prefilled syringe to treat moderate to severe persistant allergic asthma and chronic urticaria	SC	2/13/2021
Tagrisso	osimertinib	AstraZeneca	epidermal growth factor receptor antagonist	Non-small cell lung cancer	Adjuvant treatment of patients with early-stage (IB, II and IIIA) epidermal growth factor receptor-mutated (EGFRm) non-small cell lung cancer (NSCLC) after complete tumour resection with curative intent	PO	2/15/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Enhertu	fam-trastuzumab deruxtecan-nxki	AstraZeneca/ Daiichi Sankyo	HER2-directed antibody and topoisomerase inhibitor conjugate	Gastric or gastroesophageal junction adenocarcinoma	Treatment of patients with HER2-positive metastatic gastric or gastroesophageal junction (GEJ) adenocarcinoma	IV	2/15/2021
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody	Renal cell carcinoma	In combination with Cabometyx (cabozantinib) for patients with advanced renal cell carcinoma (RCC)	IV	2/20/2021
Cabometyx	cabozantinib	Exelixis	Kinase inhibitor	Renal cell cancer	In combination with Opdivo (nivoluab) for first-line treatment of advanced or metastatic renal cell cancer	PO	2/20/2021
Libtayo	cemiplimab-rwlc	Sanofi	programmed death ligand-1 (PD-L1) inhibitor	Non-small cell lung cancer	Treatment of patients with first-line locally advanced or metastatic non-small cell lung cancer (NSCLC) with ≥ 50% PD-L1 expression	IV	2/28/2021
Entresto	valsartan/ sacubitril	Novartis	Angiotensin-receptor/ neprilysin inhibitor (ARNI)	Heart failure with preserved ejection fraction	To reduce the risk of cardiovascular death and hospitalization for heart failure in patients with chronic heart failure (New York Heart Association [NYHA] Class II-IV) and preserved ejection fraction	PO	2/28/2021
Gavreto	pralsetinib	Blueprint Medicines/ Genentech	RET inhibitor	Thyroid cancer	Treatment of patients with advanced or metastatic RET-mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancer	PO	2/28/2021
Yescarta	axicabtagene ciloleucel	Kite/ Gilead	chimeric antigen receptor (CAR) T cell therapy	non-Hodgkin lymphoma	Treatment of relapsed or refractory follicular lymphoma and marginal zone lymphoma after two or more prior lines of systemic therapy.	IV	3/4/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Darzalex Faspro	daratumumab and hyaluronidase-fihj	Janssen/ Halozyme Therapeutics	humanized anti-CD38 monoclonal antibody	Light chain amyloidosis	Treatment of patients with light chain amyloidosis (AL)	SC	3/10/2021
Xpovio	selinexor	Karyopharm Therapeutics	selective inhibitor of nuclear export	Multiple myeloma	Treatment for patients with multiple myeloma after at least one prior line of therapy	PO	3/20/2021
Exparel	bupivacaine (liposomal suspension)	Pacira	local anesthetic	Analgesia	Single-dose infiltration in adults and pediatric patients 6 years and over, to produce postsurgical local analgesia and as an interscalene brachial plexus nerve block to produce postsurgical regional analgesia	INJ	3/22/2021
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Breast cancer	Treatment of patients with high-risk early-stage TNBC, in combination with chemotherapy as neoadjuvant treatment, and then as a single agent as adjuvant treatment after surgery	IV	3/29/2021
Botox	onabotulinumtoxinA	Allergan	botulinum toxin analog	Detrusor overactivity	Treatment of signs and symptoms of detrusor overactivity associated with an underlying neurologic condition (eg, spina bifida, spinal cord injuries) in pediatric patients (5 -17 years of age)	IM	3/30/2021
Rinvoq	upadacitinib	AbbVie	janus associated kinase (JAK) inhibitor	Psoriatic arthritis	Treatment of adult patients with active psoriatic arthritis	PO	4/1/2021
Rinvoq	upadacitinib	AbbVie	janus associated kinase (JAK) inhibitor	Ankylosing spondylitis	Treatment of adult patients with active ankylosing spondylitis.	PO	4/1/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Nuplazid	pimavanserin	Acadia	5-HT-2A receptor agonist	Dementia-related psychosis	Treatment of hallucinations and delusions associated with dementia-related psychosis (DRP)	PO	4/3/2021
Praluent	alirocumab	Sanofi/ Regeneron	PCSK9 inhibitor	Hyperlipidemia	Treatment of LDL-C reduction in adult patients with homozygous familial hypercholesterolemia (HoFH)	SC	4/4/2021
Tyvaso	treprostinil	United Therapeutics	prostacyclin analog	Pulmonary hypertension	Treatment of pulmonary hypertension associated with interstitial lung disease (PH-ILD).	INH	4/17/2021
Ibsrela	tenapanor	Ardelyx	sodium-hydrogen exchanger-3 (NHE-3) inhibitor	Hyperphosphatemia	To control serum phosphorus in adult patients with chronic kidney disease (CKD) on dialysis	PO	4/29/2021
Xtandi	enzalutamide	Astellas	androgen receptor inhibitor	Prostate cancer	Label update to include overall survival data from the phase 3 PROSPER study in nonmetastatic castration-resistant prostate cancer	PO	4/30/2021
Xeljanz	tofacitinib	Pfizer	Janus associated kinase (JAK) inhibitor	Axial spondyloarthritis	Treatment of axial spondyloarthritis	PO	5/15/2021
Benlysta	belimumab	GlaxoSmithKline	B-lymphocyte stimulator (BLyS)-specific inhibitor	Lupus nephritis	Lupus nephritis	IV	5/31/2021
Nuzyra	omadacycline	Paratek	tetracycline	Community-acquired pneumonia	Oral-only dosing for the treatment of community-acquired pneumonia	PO	5/31/2021
Nurtec ODT	rimegepant	Biohaven	calcitonin gene-related peptide (CGRP) inhibitor	Migraine prophylaxis	Preventive treatment of migraine in both episodic and chronic migraine patients	PO	6/1/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Shingrix	zoster vaccine recombinant, adjuvanted	GlaxoSmithKline	vaccine	Herpes zoster	Prevention of herpes zoster in adults aged 18 years and older at increased risk of herpes zoster	IM	6/15/2021
Nucala	mepolizumab	GlaxoSmithKline	IL-5 antagonist monoclonal antibody	Nasal polyps	Treatment of chronic rhinosinusitis with nasal polyposis	SC	6/15/2021
Cosentyx	secukinumab	Novartis	IL-17 receptor antagonist	Pediatric psoriasis	Treatment of pediatric psoriasis	SC	6/27/2021
Rinvoq	upadacitinib	AbbVie	janus associated kinase (JAK) inhibitor	Atopic dermatitis	Treatment of adults and adolescents with moderate to severe atopic dermatitis	PO	8/19/2021
Xarelto	rivaroxaban	Janssen	factor Xa inhibitor	Peripheral arterial disease	Reduce the risk of major thrombotic vascular events such as heart attack, stroke and amputation in patients after recent lower-extremity revascularization in patients with peripheral arterial disease (PAD)	PO	8/26/2021
Darzalex Faspro	daratumumab and hyaluronidase-fihj	Janssen/ Halozyme Therapeutics	humanized anti-CD38 monoclonal antibody	Multiple myeloma	In combination with pomalidomide and dexamethasone (D-Pd) for the treatment of patients with relapsed or refractory multiple myeloma who have received at least one prior line of therapy	SC	9/12/2021

IM = intramuscular, INH = inhaled, IV = intravenous, OPH = ophthalmic, PO = oral, SC = subcutaneous, TOP = topical

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