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4TH QUARTER 2020
PIPELINE REPORT

Be "in the know". Confidio is committed to helping clients anticipate pharmaceutical trends and potential shifts in medication cost management. Confidio remains informed of the latest FDA drug approvals, medications in development, and upcoming specialty/biosimilar pipeline therapies.

Confidio delivers client solutions that are meaningful, measurable, and sustainable.

Look for these Drug Pipeline Reports each quarter.

PIPELINE HIGHLIGHTS:

Recent Approvals

- **Zepzelca (lurbinectedin)** – Small cell lung cancer, relapsed. Approved 6/15/2020
- **Inqovi (cedazuridine + decitabine)** – Leukemia and sometimes-pre-cancerous bone marrow condition. Approved 7/7/2020
- **Tecartus (brexucabtagene)** – Cancer: Mantle cell lymphoma. Approved 7/24/2020
- **Monjuvi (tafasitamab-cxix)** – Cancer: Lymphoma. Approved 7/31/2020
- **Blenrep (belantamab)** – Blood cancer: multiple myeloma. Approved 8/5/2020
- **Evrysdi (risdiplam)** – Neuromuscular condition: Spinal muscular atrophy. Approved 8/7/2020
- **Viltepso (viltolarsen)** – Duchenne Muscular Dystrophy. Approved 8/12/2020
- **Enspryng (satralizumab)** – Autoimmune central nervous system disease: Neuromyelitis optica spectrum disorder. Approved 8/14/2020
- **Kesimpta (ofatumumab)** – Relapsing multiple sclerosis. Approved 8/20/2020
- **Gavreto (pralsetinib)** – Cancer: non-small cell lung cancer. Approved 9/7/2020

  Note: *FDA declined to approve Viaskin Peanut on 8/4/2020 due to effectiveness concerns*

Anticipated FDA Approvals

- **Ryoncil (remestemcel-L)** – Bone marrow/stem cell transplant side effect: Graft versus host disease. Expected FDA decision: 9/30/2020
- **Zokivny (lonafarnib)** - Viral liver disease: Hepatitis D. Expected FDA decision: 11/20/2020
- **Danyelza (naxitamab)** – Nervous system cancer: Neuroblastoma. Expected FDA decision: 11/20/2020
- **setmelanotide (aka RM-493)** – Obesity: due to genetic defects. Expected FDA decision: 11/27/2020
- **lumasiran (aka ALN-GO1)** - Kidney disease: primary hyperoxaluria type 1. Expected FDA decision: 12/3/2020
- **berotralstat (aka bcx7353)** – Hereditary Angioedema (HAE). Expected FDA decision: 12/3/2020
- **tanezumab** - Osteoarthritis pain. Expected FDA decision: 12/2020
- **dostarlimab (aka TSR-042)** - Cancer: endometrial. Expected FDA decision: 4Q2020

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PIPELINE HIGHLIGHTS:

Anticipated FDA Approvals Cont.

- **umbralisib (aka TGR-1202)** – Cancer: Marginal zone lymphoma. Expected FDA decision: 2/15/2021
- **trilaciclib** - Prevention of chemotherapy-induced immune suppression. Expected FDA decision: 2/15/2021
- **ropeginterferon alfa-2b** – Blood cancer: Polycythaemia vera. Expected FDA decision: 2/2021
- **aducanumab (aka BIIB037)** – Alzheimer's disease. Expected FDA decision: 3/7/2021
- **Fotivda (tivozanib)** – Kidney cancer. Expected FDA decision: 3/31/2021
- **ponesimod (aka ACT 128800)** – Multiple sclerosis. Expected FDA decision: 1Q2021

Recent FDA Approvals

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
6/15/2020	**$155,160** **Assistance for out-of-pocket costs** provided for eligible patients (subject to annual maximum) through JazzCares **Medical benefit**	**Zepzelca (fka Zepsyre)** lurbinectedin **PharmaMar Jazz**	**Lung Cancer**: Small cell lung cancer (SCLC) relapsed after platinum-containing therapy	Intravenous Small cell lung cancer is highly associated with smoking, occurs mainly in those over age 65, is slightly more common in men, and compared to most other cancers, carries a poorer prognosis. About 30,000 new cases of SCLC and 17,600 deaths are expected in 2020. **FDA Designations**: Accelerated Approval, Priority review, Orphan Drug	Alkylating agent that is a DNA minor groove binder; inhibits the growth of factors that promote tumor growth, and the production of proteins the tumor cells need to survive. SCLC incidence and death rate are both dropping due to more people quitting smoking. No new products for treatments of SCLC have been approved in more than 20 years.	**Therapy alternatives** **Topotecan**: $88,149 **Place in therapy**: Unclear. Continued approval contingent upon confirmatory trials. Non-comparative trials suggest similar efficacy to topotecan, but may be preferred due to shorter treatment window.
7/7/2020	$97,435/year **Co pay Assistance** may be available to eligible, privately insured patients through the Taiho Oncology Patient Support Program **Pharmacy Benefit**	**Inqovi** cedazuridine + decitabine (aka ASTX727) **Astex Otsuka**	**Leukemia and sometimes-pre-cancerous bone marrow condition**: myelodysplastic syndrome (MDS)--certain subtypes	Oral The number of people with MDS in the US is estimated to be about 10,000 by some; others believe it to be much higher. MDS is uncommon before age 50, and the risk increases as a person gets older. It is most commonly diagnosed in people in their 70s. **FDA Designations**: Orphan Drug, Priority Review	Antimetabolite/DNA hypomethylating agent Cedazuridine is included to allow oral administration of decitabine, by inactivating enzymes that would otherwise break it down.	**Therapy alternatives** > IV decitabine, $10,450-62,694/yr > azacytidine, $11,739-46,956/yr **Place in therapy**: Definite advantage due to oral dosing; active ingredient is the same as current standard of therapy. Has been shown to achieve as high a blood concentration as IV decitabine.

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
7/24/2020	**$373,000** **Assistance and information** on the manufacturing, shipment, insurance benefits, and travel support is available through Kite Konnect **Medical benefit**	**Tecartus** brexucabtagene autoleucel **Gilead Kite Pharma**	**Lymphoma (cancer)**: Mantle cell lymphoma (MCL), relapsed or refractory	Intravenous; single dose Will be dispensed only through hospitals and most infusions will occur while the patient is in hospital. Most serious side effect, common to infusions of immune cells, is cytokine release syndrome (CRS), occurring in 18% of patients. It can cause fever and multiple organ dysfunction that can be life-threatening. MCL comprises 2-10% of the 75,000+ cases of non-Hodgkin lymphoma diagnosed annually in the US. FDA Designations: Accelerated approval, FDA Breakthrough Therapy, Orphan Drug	**Biologic product** Chimeric antigen receptor T-cell (CAR-T) immunotherapy --the first CAR T-cell therapy approved for MCL. To prepare, a patient's own immune cells (T cells) are collected and genetically modified in a laboratory. Once infused back into the patient they find cells that express the antigen CD19 (found on MCL cells and their normal B-cell counterparts) and activate a chain of events that will kill the tumor cells. Available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Must be administered at a certified facility that monitors patients for 7 days after administration.	**Therapy alternatives** > Imbruvica $209,820 > Calquence $171,108 > Revlimid $181,392 > Venclexta $149,650 **Place in therapy**: Unclear. There is no cure for MCL, and studies comparing Tecartus to current standard of therapy have not been done. Infusion reactions can be severe, and services associated with administration can be costly.
7/31/2020	Monjuvi: $178,200 Lenalidamide: $192,276 Total annual regimen cost: **$370,476** **Copay assistance** available through the My MISSION Support Program. **Medical benefit**	**Monjuvi** tafasitamab-cxix **MorphoSys Xencor**	**Lymphoma (cancer)**: Diffuse large B cell lymphoma (DLBCL); in combination with lenalidomide for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) who are not eligible for autologous stem cell transplant (ASCT).	Intravenous DLBCL comprises about ¼ of the 75,000+ cases of non-Hodgkin lymphoma diagnosed annually in the US. It is curable in about 50% of cases, but those who fail first-line therapy have a poor prognosis. Will be available through just one specialty pharmacy (not identified yet). **FDA Designations**: Accelerated approval	**Biologic product** Anti-CD19 antibody Dosing frequency is relatively high, especially at the start of therapy, when 5 doses must be administered in 22 days.	**Therapy alternatives** > Gemcitabine and oxaliplatin, $20,000-$68,000/yr OR > Polatuzumab vedotin + bendamustine + rituximab $290,262/year **Place in therapy**: Continued approval contingent upon verification of clinical benefit, and high dosing frequency is a disadvantage. However, lack of a current standard of therapy for these patients may place Monjuvi on par with current treatments.

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
08/05/2020	**$251,068** **Copay assistance** available through Together with GSK Oncology program. **Medical benefit**	**Blenrep** belantamab mafodotin-blmf **GSK Seattle Genetics**	**Blood cancer:** Multiple myeloma, relapsed, refractory, no longer responsive to immunomodulators	Intravenous Second most common form of blood cancer in the US, it is treatable to a certain extent but not curable. Median age at diagnosis is 69 years. In 2020: > About 32,270 new cases will be diagnosed (17,530 in men, 14,740 in women). > About 12,830 deaths are expected to occur (7,190 in men, 5,640 in women). **FDA Designations:** Accelerated approval	**Biologic product** Cytotoxic agent Anti-B-cell maturation antigen (BCMA) antibody--first in class Last month, a panel of the European Medicines Agency (EMA) also recommended approval. FDA panel had earlier raised concerns about deposits on the cornea in the eyes, but later decided benefits outweigh risks. A risk evaluation and mitigation strategy (REMS) will limit administration to specially trained healthcare providers and certified healthcare facilities. Patients must be enrolled in the REMS and consent to ophthalmic exams prior to each dose.	**Therapy alternatives** Triple combinations that include daratumumab with dexamethasone and one of the following: > Bortezomib (total regimen, $199,599) OR > Carfilzomib (total regimen, $270,918) OR > Lenalidamide (total regimen, $339,072) **Place in therapy:** Continued approval contingent upon verification of clinical benefit, and potential corneal damage is a barrier to adoption. However, Blenrep's novel mechanism of action may place it on par with alternatives.
8/7/2020	**Minimum $100,000, maximum $340,000** (pricing is a sliding scale based on weight and age) (IPD Analytics, 2020) **Copay assistance** is available to eligible patients through the Genentech Patient Foundation. **Pharmacy Benefit**	**Evrysdi** risdiplam (aka RG7916) **Roche Genentech PTC Therapeutics**	**Neuromuscular condition:** Spinal muscular atrophy (SMA), types 1, 2 or 3. May be used in children as young as 2 months old.	Oral liquid Will be supplied as direct-to-patient shipments exclusively from Accredo. Risdiplam is the only SMA treatment that does not require administration by a healthcare professional. SMA affects roughly 300 infants per year in the U.S. There are 4 types. Before Spinraza (pre-12/2016), SMA was usually fatal within 2 years after birth. **FDA Designations:** Orphan drug, Fast Track, Priority Review	New drug class: SMN2 splicing modifiers. Increases the number of SMN2 genes available for making normal SMN (a protein necessary for functioning nerve cells). SMN is primarily made by the SMN1 gene, but in SMA patients, SMN1 is defective. Unlike Zolgensma (a potentially curative, one-time-dose gene therapy) but similar to Spinraza, Evrysdi manages symptoms but does not cure SMA. Evrysdi should not be administered concurrently with Spinraza.	**Therapy alternatives** > Spinraza, approved for all 4 types of SMA: $510,000 for first 60 days, then $382,500 per year > Zolgensma, approved only for type 1 SMA: $2,125,000 for one-time administration **Place in therapy:** Oral dosing, a new mechanism of action and pricing may allow Evrysdi to compete successfully with Spinraza for patients with SMA types 1, 2 and 3.

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
8/12/2020	**$733,000** for 30kg patients **Possible savings** for eligible patients on deductible, co-pay, and coinsurance through NS Support **Medical**	**Viltepso** viltolarsen (aka NS-065) **Nippon Shinyaku NS Pharma**	**Duchenne Muscular Dystrophy** (DMD) in patients with mutation amenable to exon 53 skipping	IV infusion over 60 minutes DMD is a fatal genetic neuromuscular disorder affecting one in 3,500 - 5,000 males worldwide. Second available treatment specific to DMD with mutations amenable to exon 53 skipping (about 8% of DMD patients); the other, Vyondys 53, was approved in 12/2019. Is not curative but may delay worsening of symptoms. **FDA Designations**: Priority Review, Rare Pediatric Disease, Orphan Drug, Fast Track	Masks the mutation in the DMD gene, allowing production of a functional protein in place of the one causing DMD symptoms. Approval was based on improvement in a surrogate marker. Continued approval contingent upon verification and description of clinical benefit in a confirmatory trial. A trial is already underway, but some payers consider the product investigational until FDA removes the contingency.	**Therapy alternatives** Vyondys 53 for 30kg patients, $748,800 **Place in Therapy**: Continued approval contingent upon verification of clinical benefit. As the second marketed product for this indication, it will compete directly with Vyondys 53.
8/14/2020	$220,000 for the first year, **$190,000** for subsequent years **Copay assistance may** be available through Genentech Access Solutions Ensprying Co-pay Program **Pharmacy benefit**	**Enspryng** satralizumab-mwge (aka SA237) **Roche Genentech Chugai**	**Autoimmune central nervous system disease**: Neuromyelitis optica spectrum disorder (NMOSD); in patients who have certain antibodies	Subcutaneous; monthly dosing NMOSD is rare and debilitating, sometimes causing blindness and motor dysfunction. Often misdiagnosed as multiple sclerosis. This type of NMOSD is more prevalent in women than men and in blacks than whites (in the US, prevalence in blacks, 13/100,000; in whites, 4/100,000). **FDA Designations**: Breakthrough Therapy; Orphan Drug; Priority Review	**Biologic product** Interleukin 6 receptor (IL-6R) antagonist, a monoclonal antibody that reduces the inflammation of nervous tissue that causes the symptoms. Already available in Japan.	**Therapy alternatives** > Rituximab: Biosimilar rituximab costs approximately $30,000–$37,000 > Soliris $678,381 > Uplinza $180,000–$280,000 **Place in therapy**: Self administration, novel mechanism of action and price may lead Enspryng to overtake Uplinza and Soliris in utilization; however, noncomparative studies so far suggest that rituximab may have similar efficacy, placing Enspryng second in line.

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
8/20/2020	$83,000/yr Copay assistance may be available through Alongside KESIMPTA **Pharmacy Benefit**	**Kesimpta** ofatumumab **Novartis**	**Multiple sclerosis (MS), relapsing forms**: clinically isolated syndrome, relapsing-remitting, active secondary progressive	Subcutaneous; monthly dosing MS has multiple stages that vary in intensity and duration between patients. Kesimpta is approved for all but primary progressive disease; it is approved for what is usually the first stage of MS. MS is 2-3 times more common in women than men, and nearly 1 million people in the US currently have MS.	**Biologic product** CD20-directed cytolytic antibody: binds to, and kills, immune cells that contribute to MS symptoms. Very similar to Ocrevus, except > Is self-administered, unlike Ocrevus > Not indicated for primary progressive MS as Ocrevus is > Dosed monthly vs every 6 months for Ocrevus Previously approved more than 10 years ago as an antineoplastic; however, at a much higher dose and for IV administration.	**Therapy alternatives** Ocrevus (ocrelizumab) $65,000 Aubagio (teriflunomide) $93,296 **Place in therapy**: Similar in mechanism and effectiveness to Ocrevus, Kesimpta may have better patient acceptance despite more frequent dosing due to self-administration. This also reduces therapy cost. Once generics for other MS treatments have reached a low price point, they may be required as first line therapy by payors. Tecfidera's generic currently costs more than Kesimpta; Gilenya and Aubagio generics are expected by 2024.
9/7/2020	$231,000 **Copay assistance** may be available through the GAVRETO Patient support Program administered by NeedyMeds **Pharmacy Benefit**	**Gavreto** pralsetinib (aka BLU-667) **Blueprint Medicines**	**Lung cancer**: Non-small cell lung cancer (NSCLC), RET fusion-positive	Oral RET activating gene fusions and mutations are key disease drivers in many cancer types, including NSCLC and medullary thyroid cancer. RET fusions are implicated in approximately 1 to 2 percent of patients with NSCLC. **FDA designation**: Breakthrough Therapy, Accelerated approval	Inhibits activity of cancer cell gene fusions (RET-activating gene fusions) and mutations that cause cancer symptoms. Also being studied for medullary thyroid cancer and other types of cancers associated with RET fusions/ mutations.	**Therapy alternatives** > Alecensa (alectinib) $187,318 > Retevmo (selpercatinib) $206,004-$247,200 **Place in therapy**: Unclear. Continued approval may be contingent upon confirmatory trials. Like Retevmo, is targeted therapy for RET-fusion positive cancers and therefore may be superior to older therapies; but comparative studies not done.

Anticipated FDA Approvals

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
9/30/2020	**Ryoncil** remestemcel-L **Mesoblast**	**Bone marrow/stem cell transplant side effect:** Graft versus host disease (GVHD) refractory to steroids Also has clearance from FDA for expanded access compassionate use (outside of clinical trials) for respiratory distress syndrome due to COVID-19.	IV GVHD occurs when stem cells given to a patient (as a treatment) attack the patient's healthy cells, causing damage to numerous organs and tissues. If treatment with steroids fails, mortality rate can be up to 90%. **FDA Designation:** Fast-track	**Biologic product** Stem cell therapy created by culturing human stem cells (from healthy volunteers) in a laboratory. When given to a patient with GVHD, they act to interrupt the GVHD process. There are no safe and effective therapies for children with steroid-refractory GVHD.	**Therapy alternatives** None
11/20/2020	**Zokinvy** lonafarnib **Eiger BioPharmaceuticals Merck & Co**	**Viral liver disease:** Hepatitis D **Accelerated aging conditions:** Progeria and progeroid laminopathies	Oral Hepatitis D may be acute or chronic. It is uncommon in the US but exact figures are unknown. Only occurs in people who already have hepatitis B; treatment for both may be necessary. Progeria: An estimated 400 children worldwide have progeria and progeroid laminopathies. **FDA Designations:** Hepatitis D: Orphan Drug Progeria/progeroid laminopathies: Orphan Drug, Breakthrough Therapy, Priority Review, Rare Pediatric Disease	Farnesyltransferase inhibitor: In hepatitis D, it a blocks a process in humans that is needed for viral replication. In progeria and progeroid laminopathies, lonafarib prevents a step in production of abnormal proteins otherwise produced by mutated genes. Historically, children with progeria lived to an average age of 14.5 years.	**Therapy alternatives** Hepatitis D: Interferons (not curative) Progeria: None
11/20/2020	**Danyelza** naxitamab **Y-mAbs Therapeutics**	**Nervous system cancer:** Neuroblastoma	IV infusion Occurs most often in infants and children (rare in children older than 10 years). Often starts in nerve tissue in the abdomen; may behave in many different ways. Diagnosed in about 700 children in the US each year. **FDA designation:** Priority review, Breakthrough Therapy, Orphan Drug	**Biologic product** Antibody that binds to tumor cells, triggering a response that kills tumor cells. New mechanism of action for treating this condition.	**Therapy alternatives** Surgery or therapy with octreotide or combination chemotherapy

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
11/27/2020	**Brand name TBD** setmelanotide (aka RM-493) **Rhythm Pharmaceuticals**	**Obesity**: severe obesity and insatiable hunger that result from rare genetic defects in the MC4R pathway	Subcutaneous **FDA designation**: Breakthrough Therapy, Orphan Drug	Melanocyte-stimulating hormone analog: restores activation of the MC4R pathway in the hypothalamus.	**Therapy alternatives** None
12/3/2020	**Brand Name TBD** lumasiran (aka ALN-GO1) **Alnylam Dicerna Pharmaceuticals**	**Kidney disease**: Primary hyperoxaluria type 1 (PH1)	Subcutaneous PH1 is an ultra-rare, inherited disorder resulting from a buildup of oxalate, which combines with calcium to form kidney and bladder stones. It can lead to end-stage renal disease, which may be life threatening. 1-3 million people in North America have PH1. **FDA Designations**: Priority Review, Orphan Drug, Breakthrough Therapy, and Pediatric Rare Disease	Prevents production of oxalate by blocking an enzyme. Another product less advanced in development has potential to overshadow lumasiran with better dosing and broader indications.	**Therapy alternatives** None
12/3/2020	**Brand name TBD** berotralstat (aka bcx7353) BioCryst	**Hereditary Angioedema (HAE)**: Prevention of hereditary angioedema (HAE) attacks	Oral HAE, which affects about 1 in 50,000 people, causes recurrent episodes of severe swelling in the limbs, face, intestinal tract, and airway. Swelling in the airway can cause life-threatening airway obstruction. **FDA designations**: Through a manufacturer sponsored expanded access program, physicians may be able to request berotralstat for patients ineligible for clinical trials.	Plasma kallikrein inhibitor: inhibits uncontrolled activity of kallikrein, which otherwise causes overproduction of symptom-causing bradykinin. Does not appear to be as effective as competitor product Takhzyro, may be priced lower as a result. Untreated HAE patients have, on average, a 3-4 day attack every 1 to 2 weeks.	**Therapy alternatives** > Takhzyro > Cinryze > Haegarda

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
12/2020	**Brand name TBD** tanezumab **Pfizer Eli Lilly**	**Arthritis**: Osteoarthritis (OA) pain	Subcutaneous OA is a leading cause of disability. Patients with knee or hip OA also have a higher than usual risk of death due to cardiovascular events.	**Biologic product** Antibody that blocks a chemical, nerve growth factor, involved in transmitting osteoarthritis pain. First of this type of treatment for OA. Studies showed no risk of addiction, dependence, or misuse.	**Therapy alternatives** Combination of non-pharmacological approaches and analgesics (acetaminophen non-steroidal anti-inflammatory drugs and opioids).
4Q2020	**Brand Name TBD** dostarlimab (aka TSR-042) **Tesaro GSK AnaptysBio**	**Endometrial cancer**: Recurrent or advanced mismatch repair-deficient (dMMR) endometrial cancer who progressed on or after a platinum-based regimen.	Injectable Endometrial cancer is the most common cancer of the female reproductive organs, with over 65,000 new cases and 12,600 deaths expected for 2020. dMMR is found in up to 30% of recurrent endometrial cancer. Mismatch repair mechanisms correct mistakes made within cells when DNA is copied. Cells that are mismatch repair deficient usually have many DNA mutations, some that can cause cancer.	**Biologic product** Programmed cell death 1 (PD-1) inhibitor: a "checkpoint inhibitor" that prevents interruption of an immune response that targets cancer cells. If approved, would be the 7th drug in this class. Other products include Libtayo (cemiplimab), Opdivo (nivolumab) and Keytruda (pembrolizumab). These are used for many types of cancer.	**Therapy alternatives** Pembrolizumab
02/15/2021	**Brand Name TBD** umbralisib (aka TGR-1202) **TG Therapeutics**	**Lymphoma (cancer)**: Marginal zone lymphoma (MZL)	Oral MZL is a slow-growing type of non-Hodgkin lymphoma, comprising about 8% of the 75,000+ cases of non-Hodgkin lymphoma diagnosed annually in the US. **FDA designations**: Breakthrough Therapy, Priority Review	Phosphoinositide 3-kinase (PI3K) and casein kinase-1 epsilon inhibitor There are at least 4 other PI3K inhibitors on the market, used to treat lymphomas, leukemias and breast cancer.	**Therapy alternatives** Combination regimens such as > "R-CHOP" OR > Bendamustine + obinutuzumab
02/15/2021	**Brand Name TBD** trilaciclib **G1 Therapeutics Boehringer Ingelheim**	**Prevention of chemotherapy-associated immune suppression**: in patients with small cell lung cancer being treated with chemotherapy	Intravenous Treatment for SCLC is particularly hard on the bone marrow, and can cause severe drops in red blood cells, white blood cells and/or platelets. These effects often result in the need to reduce a patient's chemo dose. **FDA designations**: Breakthrough therapy; Priority Review	CDK4/6 dual inhibitor--protects bone marrow cells that produce red blood cells, white blood cells and platelets from chemotherapy toxicity. Is unique in that it is given before toxicity begins. Also being tested in patients being treated for colorectal and breast cancer	**Therapy alternatives** > Filgrastim (e.g., Neupogen) > Pegfilgrastim (e.g., Neulasta) > Sargramostim (Leukine)

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
02/2021	**Brand name TBD** ropeginterferon alfa-2b **PharmaEssentia**	**Blood cancer**: Polycythaemia Vera (PV)	Subcutaneous, every 2 weeks PV results in too many red blood cells in the blood, thickening it and causing serious effects such as blood clots. PV develops slowly over a number of years. It is rare, affecting approximately 2 people per 100,000. It occurs most often in individuals older than 60 years. **FDA designations**: Orphan drug	**Biologic product** Pegylated interferons It appears that the effects of ropeginterferon build more slowly than those of standard therapy, and may last longer. Ropeginterferon alfa-2b was approved by the European Commission in February 2019.	**Therapy alternatives** Hydroxyurea
03/07/2021	**Brand name TBD** aducanumab (aka BIIB037) **Biogen Neurimmune**	**Alzheimer's disease (AD)**	Intravenous AD is expected to affect 13.9 million Americans or 3.3% of the country's population, by 2060. **FDA designations**: Priority review	**Biologic product** Amyloid beta protein inhibitor which attacks amyloid plaques that are thought to cause symptoms of AD. Product is the first in 17 years to be reviewed by FDA for treatment of AD. If approved, aducanumab would be the first treatment designed to delay progression of the disease Development of the product was halted at one point, following disappointing study results, but was resumed in October of 2019.	**Therapy alternatives** > Galantamine > Rivastigmine
03/31/2021	**Fotivda** tivozanib **Aveo Ophthotech Kyowa Kirin**	**Kidney cancer**: relapsed or refractory renal cell carcinoma as third- and fourth-line treatment	Oral In 2020, about 28,000 new cases of kidney cancer will be diagnosed in women, and about 74,000 cases in men. Kidney cancer is more common in African Americans and American Indian /Alaska Natives, and the average age at diagnosis is 64. Renal cell carcinoma (RCC) comprises about 9 out of 10 cases of kidney cancer.	Vascular endothelial growth factor receptor (VEGFR) tyrosine kinase inhibitor FDA declined to approve tivozanib in 2013 and when Aveo filed the new application in March 2020, the company agreed to withdraw the NDA if study results did not demonstrate an increased chance of survival compared to sorafenib. Sorafenib, however, is not one of NCCN's preferred regimens for treating kidney cancer that has relapsed.	**Therapy alternatives** > Cabozanitib > Nivolumab

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
1Q 2021	**Brand name TBD** ponesimod (aka ACT-128800) **Actelion JNJ Janssen**	**Multiple Sclerosis**: Relapsing multiple sclerosis (MS)	Oral Estimates of the prevalence of MS were updated in 2019 to reflect a figure twice as high as before, now indicating that more than 900,000 people in the US are living with MS.	Sphingosine 1-phosphate (S1P) receptor modulators The ponesimod molecule is more likely than other drugs in its class (such as Gilenya and Mayzent) to bind to the cells where they exert their effects. It was shown superior to Aubagio in reducing MS flares in a head-to-head study.	**Therapy alternatives** > Gilenya > Mayzent > Zeposia
CRL* issued on 8/18/2020	**Roctavian** valoctogene roxaparvovec **BioMarin**	**Hemophilia A**	BioMarin Pharmaceuticals CEO Jean-Jacques Bienaimé suggested that the company is considering charging $2-3 million. Given as a single lifetime dose **FDA Designations**: Priority Review; Accelerated Approval; Breakthrough Therapy; Orphan Drug. **CRL issues**: > Phase 3 study was too different from the Phase 1/2 study to support durability of effect.	**Biologic product** Uses a modified, harmless virus to deliver a working copy of the gene that is mutated in hemophilia, allowing production of the missing clotting factor. Product is potentially curative: 3 years after dosing, all 7 study participants who received the highest dose had had no bleeding events, no factor infusions needed and no bleeding into joints. FDA has also accepted premarket approval application for an AAV5 total antibody assay to be used as a companion diagnostic.	**Therapy alternatives** Symptomatic only. BioMarin estimates overall lifetime cost of care is $25 million for hemophilia patients.
CRL* issued on 8/18/2020	**Brand name TBD** filgotinib (aka GLPG0634) **Galapagos Gilead**	**Rheumatoid Arthritis**, moderate to severe	Oral **FDA Designations**: Priority Review **CRL issues**: > FDA seeking more data on testicular safety, semen parameters > Concern over risk/benefit of 200mg dose	Janus kinase (JAK1) inhibitor Claimed to potentially be better than adalimumab and safer than tofacitinib and baricitinib; in a crowded market of very expensive products.	**Therapy alternatives** > Humira (adalimumab) > Rinvoq (upadacitinib) > Xeljanz (tofacitinib) > Olumiant (baricitinib)

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Alternatives
CRL* issued 5/31/2020	**Instiladrin** nadofaragene firadenovec **FKD Therapies Merck & Co Ferring FerGene Trizell**	**Bladder cancer**, non-muscle-invasive (NMIBC) unresponsive to BCG.	Intravesical (administered into the bladder) NMIBC is cancer that only affects the first layers of the inside of the bladder. About 81,400 new cases of bladder cancer, and 17,980 deaths, are expected to occur in 2020. **FDA Designation:** Priority review **CRL issues:** FDA cited manufacturing issues as the reason behind the CRL	**Biologic product** A non-infectious virus is used to insert the drug interferon alfa 2b into tumor cells, causing the cells to die. BCG, first-line therapy for this condition, is in chronic short supply. Is given less frequently than competing products, and may be more effective.	**Therapy alternatives** > Gemcitabine or mitomycin administered into the bladder > Pembrolizumab
CRL* issued on 3/9/2020	**Brand name TBD** exenatide (aka ITCA 650) **Intarcia Therapeutics**	**Diabetes**: type 2	Implant Implanted once per year.	Exenatide has been used in treating type 2 diabetes since 2011, but must be injected at least weekly (brand names Byetta and Bydureon).	**Therapy alternatives** Standard treatment for type 2 diabetes.

*CRL (Complete Response Letter) is a communication to a drug's manufacturer from the FDA indicating that the application for the drug cannot be approved in its present form.

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The above information was assembled from government and clinical resources for knowledge purposes only. Information and drugs were selected by clinicians based on therapy and potential clinical impact without any manufacture affiliations or conflicts of interest. Approval status, dates, and WAC price are subject to variation. This document should not be exclusively used for decision-making purposes. WAC pricing data should be used for benchmarking purposes only. Prices listed above should not be used alone to set or adjudicate any prices for reimbursement or purchasing functions or considered to be an exact price for a single product and/or manufacturer.

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