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2ND QUARTER 2020
PIPELINE REPORT

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PIPELINE HIGHLIGHTS:

Recent Approvals

- **Ervebo (ebola Zaire vaccine, live) –** Prevention of Ebola. Approved 12/18/2019
- **Tazverik (tazemetostat) –** Cancer: Epithelioid sarcoma. Approved 1/23/2020
- **Tepezza (teprotumumab-trbw) –** Thyroid eye disease: Graves' orbitopathy. Approved 1/21/2020
- **Ayvakit (avapritinib, aka BLU-285) –** Cancer of the GI tract: gastrointestinal stromal tumors (GIST). Approved 1/9/2020
- **Padcev (enfortumab vedotin, aka ASG-22ME) –** Cancer of the urinary tract: urothelial cancer. Approved 12/18/2019
- **Palforzia, aka AR101 –** Peanut allergy. Approved 1/31/2020
- **Vyondys 53 (golodirsen, aka SRP-4053) –** Duchenne Muscular Dystrophy. Approved 12/12/2019
- **Sarclisa (isatuximab-irfc) –** Blood cancer: Multiple myeloma. Approved 3/2/2020

Anticipated FDA Approvals

- **Exenatide implant –** Diabetes: type 2. Expected FDA decision: 3/9/2020
- **Risdiplam (aka RG7916) –** Neuromuscular condition: Spinal muscular atrophy. Expected FDA decision: 5/24/2020
- **Pemigatinib (aka INCB054828) –** Cancer of the bile duct (cholangiocarcinoma). Expected FDA decision: 5/30/2020
- **Sacituzumab govitecan-hziy (aka IMMU-132) –** Breast cancer, triple negative. Expected FDA decision: 6/2020
- **Inebilizumab –** Autoimmune central nervous system disease: Neuromyelitis optica spectrum disorder. Expected FDA decision: 6/2020
- **Selumetinib sulphate –** Neurological condition: neurofibromatosis type 1. Expected FDA decision: 2Q2020

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
12/18/2019	**$379,800** Padcev Copay Assistance Program available. Medical benefit	**Padcev** (enfortumab vedotin; aka ASG-22ME) Seattle Genetics Astellas	**Cancer of the urinary tract:** Urothelial cancer in patients who have failed certain other treatments.	IV May be a breakthrough for resistant urothelial cancer, which progresses in 75-80% of patients even after treatment with currently preferred agents. >50% of patients experienced neuropathy, and 6% stopped therapy for this reason. **FDA Designations:** Accelerated Approval	First-in-class monoclonal antibody/ drug conjugate that targets Nectin-4, a protein located on cell surfaces that is highly expressed in bladder cancer	**Therapy alternatives:** Preferred: Balversa $306,979
1/9/2020	**$384,000** YourBlueprint Copay Assistance Program available. Pharmacy benefit	**Ayvakit** (avapritinib; aka BLU-285) Blueprint Medicines	**Cancer of the GI tract:** gastrointestinal stromal tumors (GIST) with PDGFRA exon 18 mutation	Oral; must be taken on an empty stomach Incidence of GIST in the US is about 0.68 per 100,000 people. Approximately 6% of newly diagnosed GIST has the type of mutation this drug is approved for. **FDA Designations:** Priority Review, Breakthrough Therapy, Fast Track, Orphan Drug	Tyrosine kinase inhibitor (TKI). Selectively targets molecules that drive tumor growth. There is no previously available treatment for the cell mutation that avapritinib targets; this type of GIST progresses within 3-4 months after the previously preferred treatment for metastatic disease.	**Therapy alternatives:** Imatinib $112,420

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
1/21/2020	**$342,700** Copay assistance available through Horizon Patient Services Program Medical Benefit	**Tepezza** (teprotumumab-trbw) Horizon Pharma Roche Genmab	**Thyroid eye disease (TED):** Graves' orbitopathy	IV In people with hyperthyroidism, thyroid eye disease causes the eyes to "bulge out" and/or double vision. There is currently no good treatment for this aside from symptomatic treatment. Patients receive a dose every 3 weeks for 8 doses only. Therapy must be given during the 1-3 year window of active disease. **FDA Designations:** Breakthrough Therapy, Fast Track, Orphan Drug	Human monoclonal antibody of the insulin-like growth factor type-1 receptor. Prevents the antibodies causing the condition from attaching to the cells they attack. Target population: 15-20,000 patients for this indication.	**Therapy alternatives:** None
1/23/2020	**$186,000** EpizymeNOW Copay Assistance Program available, complimentary supplies for those with coverage delays and emergency supplies Pharmacy benefit	**Tazverik** (tazemetostat) Epizyme	**Cancer:** Epithelioid sarcoma, metastatic or locally advanced and not eligible for surgical treatment	Oral Also being studied for previously treated relapsed/refractory follicular lymphoma Approximately 800 people in the US have this condition; 300 may be eligible for treatment. **FDA Designations:** Priority Review; Orphan Drug. Approval contingent on verification of clinical benefit.	Inhibitor of enhancer of zeste homolog 2 (EZH2); first drug in its class. Decreases tumor cell proliferation by Inhibiting an enzyme that is too abundant or is defective in many types of cancer. Has (rarely) caused other types of cancer.	**Therapy alternatives:** Surgery and/or radiation therapy

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
12/18/2019	**TBD** Medical benefit	**Ervebo** (ebola Zaire vaccine, live, aka V920 and rVSV-ZEBOV-GP) Merck & Co NewLink Genetics	**Prevention of ebola**	Intramuscular Ebola is highly contagious and often fatal. Is 100% effective for the Zaire ebola strain but not effective for other ebola strains or the Marburgvirus; onset and duration of immunity are unknown. **FDA Designations:** Priority Review; Breakthrough Therapy; Tropical Disease Priority Review voucher.	Has been available as of August 2019 to health-care workers on a "compassionate use" basis; found to 97.5% effective for those vaccinated vs. those not vaccinated. Merck & Co (MRK.N) plans to launch in the third quarter of 2020 and price at the lowest possible access price for poor and middle-income countries.	**Therapy alternatives:** None
1/31/2020	**$11,000** Support and financial options through Palforzia Pathway. Pharmacy benefit	**Palforzia (AR101)** Aimmune	**Peanut allergy**	Oral powder Peanut allergy is common in children, with an incidence of approximately 1.4-4.5% Will be available through specially certified providers enrolled in the REMS program. FDA requires providers be educated on risk of anaphylaxis. **FDA Designations:** Breakthrough Therapy; Fast Track	Uses gradually increasing doses of peanut protein to desensitize patients. Patients must continue to avoid peanuts. ICER evaluation predicted cost-effectiveness if priced as expected and concluded that evidence for improved outcomes with AR101 compared to peanut avoidance alone was promising but inconclusive.	**Therapy alternatives:** •Peanut avoidance •Viaskin (transdermal patch currently in development)

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
1/12/2019	**$300,000** Financial assistance through SareptAssist. Medical benefit	**Vyondys 53** golodirsen (aka SRP-4053) Sarepta Therapeutics	**Duchenne Muscular Dystrophy (DMD)** in patients with mutation amenable to exon 53 skipping	IV DMD is a fatal genetic neuromuscular disorder affecting one in 3,500 - 5,000 males born worldwide. Golodirsen targets DMD with a specific genetic mutation that affects about 8% of DMD patients. It is not curative but may delay worsening of symptoms. **FDA Designations:** Priority Review; Accelerated Approval. Continuing approval contingent on verification of clinical benefit.	Masks the mutation caused by the DMD gene, allowing production of a functional protein in place of the one causing DMD symptoms. FDA had previously rejected this product due to concerns about possible kidney damage.	**Therapy alternatives:** •Emflaza (deflazacort) $185,000 (oral) •Prednisone generic, $115-$1000 Emflaza improves symptoms, similar to prednisone; Vyondys 53 directly addresses the underlying disease process.
3/2/2020	**$135,200** (must give with pomalidomide and dexamethasone, for a total annual therapy cost of $373,930) Financial Assistance through CareASSIST Medical benefit	**Sarclisa** (isatuximab-irfc, aka SAR650984) Sanofi ImmunoGen	**Blood cancer:** Relapsed, refractory multiple myeloma	IV Multiple myeloma relapses repeatedly, making it essentially incurable. Sarclisa will be distributed through a limited network **FDA Designations:** Orphan Drug.	Monoclonal antibody that targets a relatively unique feature of myeloma cells, causing them to die and also activating the immune system to kill these cells.	**Therapy alternatives:** Lenalidomide + dexamethasone + bortezomib $280,350

Anticipated FDA Approvals

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
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.
5/24/2020	**Brand Name TBD** (risdiplam, aka RG7916) Roche Genentech PTC Therapeutics	**Neuromuscular condition** Spinal muscular atrophy (SMA), types 1, 2 or 3.	Oral liquid If approved, risdiplam will be the only SMA treatment that does not require administration by a healthcare professional. Before Spinraza (pre-12/2016), SMA was usually fatal within 2 years. **FDA Designations:** Orphan drug, Fast Track, Priority Review	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. Roche plans to price risdiplam at a discount to both Zolgensma and Spinraza.	**Therapy alternatives:** • Spinraza (nusinersen), approved for all 4 types of SMA • Zolgensma (onasemnogene abeparvovec-xioi), approved only for type 1 SMA
5/30/2020	**Brand name TBD** pemigatinib (aka INCB054828) Incyte	**Cancer of the bile duct** (cholangiocarcinoma), second-line for locally advanced or metastatic	Oral This type of cancer is often not diagnosed until prognosis is poor. **FDA Designation:** Priority Review.	Fibroblast growth factor receptor (FGFR) inhibitor—reduces changes in FGFRs that are associated with cancer development. This would be the first targeted therapy for this type of cancer.	**Therapy alternatives:** None First-line therapy for biliary tract cancer is combination cisplatin + gemcitabine. There is no standard second-line therapy.
6/2020	**Brand Name TBD** (sacituzumab govitecan-hziy ; aka IMMU-132) Immunomedics	**Breast cancer:** Refractory, metastatic hormone receptor triple negative	IV This subtype of breast cancer has very low response rate and short survival time following standard therapy; the survival rate has not improved in the last 20 years.	Monoclonal antibody/drug conjugate that targets specific tumor cells.	**Therapy alternatives:** • Cyclophosphamide/ doxorubicin + paclitaxel • Cyclophosphamide/ docetaxel

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
6/2020	**Brand Name TBD** (inebilizumab) AstraZeneca MedImmune Viela	**Autoimmune central nervous system disease:** Neuromyelitis optica spectrum disorder (NMOSD)	IV NMOSD is rare and debilitating, sometimes causing blindness and motor dysfunction. It is often misdiagnosed as multiple sclerosis. NMOSD is more prevalent in women than men **FDA Designations:** Breakthrough Therapy; Orphan Drug.	Anti-CD19 antibody: Monoclonal antibody that depletes the overabundance of immune cells which contributes to this condition. Second product approved for this indication in 2019 (Soliris was approved in June 2019). Prior to that, treatments were symptomatic only.	**Therapy alternatives:** Rituximab Soliris (eculizumab)
2Q2020	**Brand name TBD** selumetinib sulphate Array BioPharma Pfizer Astrazeneca Merch & Co	**Neurological condition:** neurofibromatosis type 1 (NF1)	Oral NF1 is an incurable, inherited disease. It is a serious condition that causes growths on skin, tumors that grow along nerves, and numerous other painful, disfiguring and life-threatening effects. **FDA Designations:** Breakthrough Therapy; Orphan Drug; Priority Review	MEK 1/2 inhibitor: inhibits the MEK enzyme, which can become dysregulated in people with NF gene mutations and cause uncontrolled cell replication. Being tested for use in children at least 3 years old. Has been tested for cancer indications but not shown significant enough activity.	**Therapy alternatives:** None - Symptomatic only (including surgical removal where possible)
8/5/2020	**Viaskin Peanut** Generic name TBD DBV Technologies	**Peanut allergy**	Topical—transdermal patch	Allergen immunotherapy Indicated for children aged 4-11	**Therapy alternatives:** Palforzia (oral allergen powder-dnfp), approved 1/31/2020
8/13/2020	**Brand name TBD** ripretinib (aka DCC-2618) Deciphera	**GI Tract cancer,** gastrointestinal stromal tumor (GIST)	Oral **FDA Designations:** Fast Track; Priority Review; Breakthrough Therapy	Broad-spectrum KIT and PDGFRa inhibitor—inhibits mutations in the KIT gene associated with GIST	**Therapy alternatives:** Ayvakit (avapritinib)
8/20/2020	**Brand name TBD** tucatinib Seattle Genetics	**Breast cancer,** HER2-positive, locally advanced unresectable or metastatic, in patients who have had at least 3 HER2-directed treatments.	Oral **FDA Designation:** Breakthrough Therapy	Tyrosine kinase inhibitor To be used in combination with trastuzumab (Herceptin) and capecitabine	**Therapy alternatives:** Pertuzumab + trastuzumab + a taxane (docetaxel or paclitaxel)

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
8/21/2020	**Valrox** (valoctogene roxaparvovec) BioMarin	**Hemophilia A Gene Therapy**	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.	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.
8/2020	**Brand name TBD** cedazuridine + decitabine (aka ASTX727) Astex Otsuka	**Leukemia and sometimes-pre-cancerous bone marrow condition:** myelodysplastic syndrome, intermediate and high risk; and chronic myelomonocytic leukemia (CMML)	Oral **FDA Designation:** Orphan Drug	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 or azacitidine
8/2020	**Brand name TBD** satralizumab (aka SA237) Roche Chugai	**Autoimmune central nervous system disease:** Neuromyelitis optica spectrum disorder (NMOSD)	Subcutaneous Monthly dosing NMOSD is rare and debilitating, sometimes causing blindness and motor dysfunction. It is often misdiagnosed as multiple sclerosis. NMOSD is more prevalent in women than men.	Interleukin 6 receptor (IL-6R) antagonist, a monoclonal antibody that reduces the inflammation of nervous tissue that causes the symptoms.	**Therapy alternatives:** Rituximab Soliris (eculizumab)
8/2020	**Brand name TBD** capmatinib (aka INC280) Novartis Incyte	**Lung cancer,** non-small cell (NSCLC), first-line and previously untreated locally advanced or metastatic METex14 mutated.	Oral The METex14 mutation is found in about 3-4% of patients with NSCLC, most of whom are older. **FDA Designations:** Breakthrough Therapy; Priority Review	C-Met inhibitor—inhibits functions associated with the c-Met protein. cMet is abnormal in many tumor types, and if not inhibited tends to promote tumor growth. First targeted agent for NSCLC with a MET exon 14 skipping mutation, which is associated with aggressive disease and a poor prognosis	**Therapy alternatives:** None (targeted therapy is used in this situation)

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
3Q 2020	**Brand name TBD** selpercatinib (aka LOXO-292) Loxo Oncology Eli Lilly	**Lung and thyroid cancers:** advanced RET fusion-positive non-small cell lung cancer (NSCLC); RET-mutant medullary thyroid cancer (MTC); and RET fusion-positive thyroid cancer.	Oral **FDA Designations:** Breakthrough Therapy; Orphan Drug; Priority Review	RET inhibitor—inhibits an enzyme that can cause tumors when mutated.	**Therapy alternatives:** Vandetinib or cabozantinib
3Q 2020	**Brand name TBD** filgotinib (aka GLPG0634) Galapagos Gilead	**Rheumatoid Arthritis**, moderate to severe	Oral **FDA Designation:** Priority Review	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)
CRL* issued on 03/22/2019	**Zynquista** sotagliflozin Lexicon Phamaceuticals	**Diabetes:** For patients with type 1 diabetes who lack adequate blood sugar control on insulin therapy.	Oral Used along with insulin	Diabetic ketoacidosis, a serious diabetes complication, was more frequent in those given sotagliflozin compared to insulin alone. In January 2019, the FDA Advisory Committee were evenly divided on whether benefits of Zynquista outweighed risks. Received approval in Europe 8/9/2019. On July 26, 2019, Sanofi announced discontinuation of their collaboration with Lexicon in developing Zynquista following release of results from 3 clinical trials.	**Therapy alternatives:** Insulin
CRL* issued 12/5/2019	**Brand name, generic name TBD** RVT-802 Enzyvant Roivant	**Primary immunodeficiency**, congenital athymia (complete DiGeorge Syndrome, cDGS)	Implant cDGS is a rare pediatric condition, uniformly fatal if untreated (usually within 2 years due to infection) **FDA Designations:** Breakthrough Therapy; Regenerative Medicine Advanced Therapy; Orphan Drug; Priority Review; Rare Pediatric Disease	Tissue-based regenerative therapy comprised of specially-treated cells that are implanted into muscle; the result is production of functioning immune cells. CRL is based on issues raised by FDA about the product's manufacturing process and site.	**Therapy alternatives:** Thymic or hematopoietic cell transplantation (HCT)

*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 manufacturer 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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