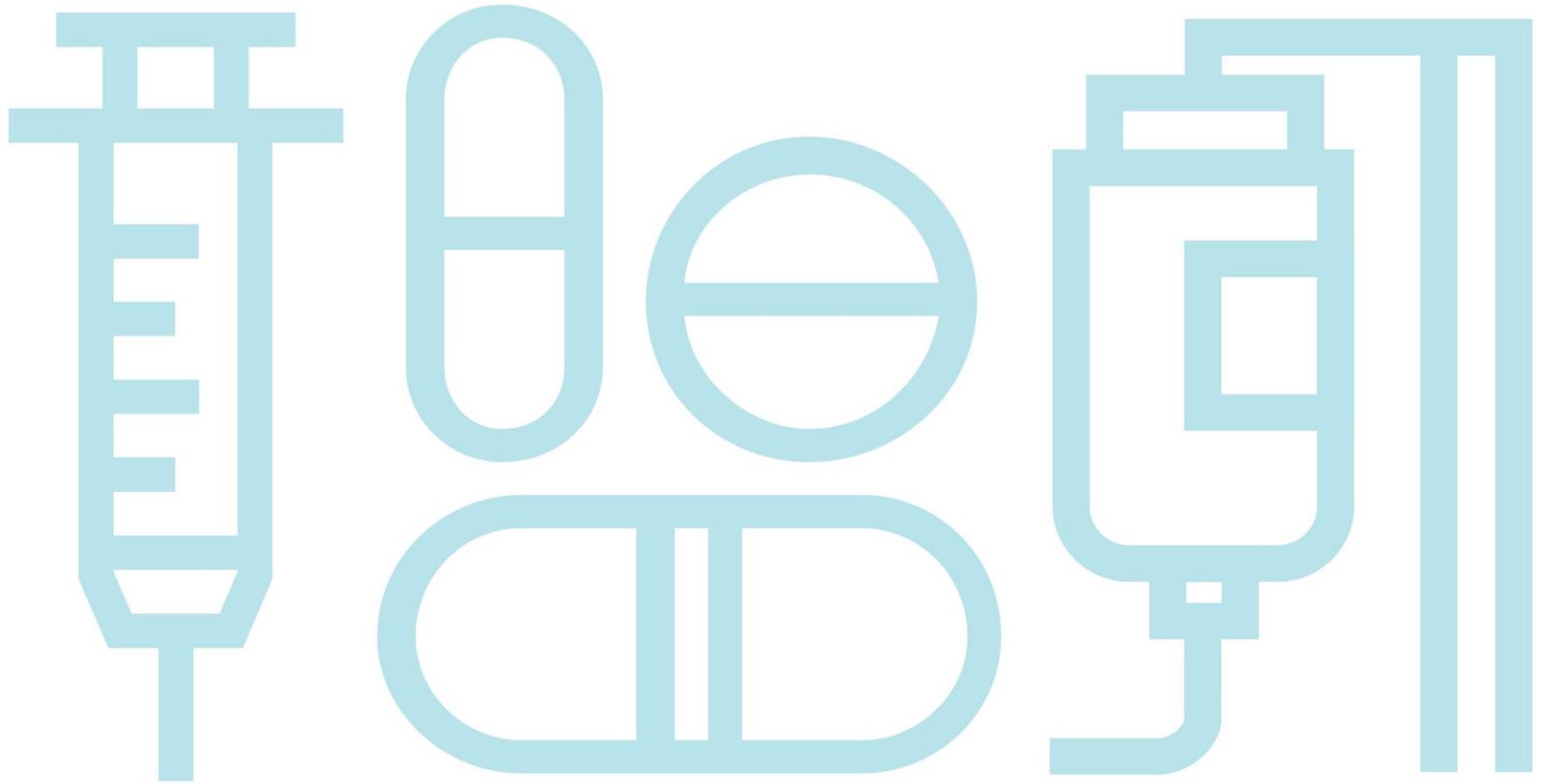


# Specialty Pharmacy Pipeline

## Drugs to Watch

Anticipated Launches | Q4 2019 – Q1 2020

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Therapeutic Category	Product Name, Route of Administration and Manufacturer <sup>1</sup>	Proposed Indication <sup>1</sup>	Phase of Study <sup>1</sup>	Disease Prevalence and Background	Select Available U.S. Food and Drug Administration (FDA) Approved Therapies	Comments
Anemia	luspatercept subcutaneous (SC) injection  Celgene/ Acceleron Pharma	The treatment of beta-thalassemia-associated anemia in adult patients who require red blood cell transfusions	Pending FDA approval 12/04/2019	Beta-thalassemia is an inherited blood disorder that reduces production of hemoglobin, an important component of red blood cells responsible for carrying oxygen throughout the body. Beta-thalassemia can lead to life-threatening anemia (decreased red blood cells). Chronic blood transfusions can be required but may lead to organ damage and even death due to iron overload. <sup>2</sup>  Beta-thalassemia is relatively rare in the U.S. with an estimated prevalence of 1,500 cases. <sup>3</sup>	None	Luspatercept will provide the first therapy for beta thalassemia that can reduce the need for blood transfusions. It will be included in Specialty Guideline Management.  <i>Anticipated impact: New spend</i>
Cystic Fibrosis (CF)	elexacaftor/ tezacaftor/ ivacaftor oral  Vertex Pharmaceuticals	The treatment of CF in patients 12 years and older with two copies (homozygous) of the cystic fibrosis transmembrane conductance regulator (CFTR) F508del mutation or with 1 copy of the CFTR-F508del mutation and a minimal function CFTR mutation (heterozygous)	Pending FDA approval 03/19/2020	CF is a progressive genetic disease that causes the mucus in various organs to become thick and sticky as a result of a dysfunctional protein in the body, known as the CFTR protein. In the lungs, mucus clogs the airways, leading to persistent infections and inflammation. In the pancreas, buildup of mucus can prevent the release of digestive enzymes that help the body absorb food and key nutrients, resulting in malnutrition and poor growth. <sup>4</sup>  There are an estimated 30,000 CF patients in the U.S. The most common CFTR mutation is F508del with 45.3% of CF patients having homozygous mutation and an additional 40.9% that are heterozygous. <sup>5</sup>	<b>CFTR modulator agents:</b> Kalydeco (ivacaftor), Orkambi (lumacaftor/ivacaftor), Symdeko (tezacaftor/ivacaftor)	The elexacaftor, tezacaftor and ivacaftor treatment regimen was granted Breakthrough Therapy designation and will provide the first disease modifying drug therapy for CF patients with heterozygous F508del mutation. It will be included in Specialty Guideline Management.  <i>Anticipated impact: Incremental spend</i>

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Human Immuno-deficiency Virus (HIV)	cabotegravir and rilpivirine  oral (initial 4 weeks only)/ intramuscular (IM) injection  ViiV Healthcare/ GlaxoSmith-Kline/Pfizer/ Janssen/Johnson & Johnson	The maintenance treatment of HIV-1 infection	Pending FDA approval 12/29/2019	HIV is a virus that attacks and destroys the body's immune system, specifically the CD4 cells, often called T-cells. If left untreated, HIV can significantly weaken the immune system, making a person more vulnerable to other infections and diseases. <sup>6</sup>  An estimated 1.1 million people aged 13 and older are living with HIV in the U.S., which includes approximately 14% who have not been diagnosed. Among all patients living with the disease, an estimated 63% receive some level of HIV medical care. <sup>7</sup>	<b>Complete regimen fixed-dose combinations for HIV:</b> Atripla (efavirenz/ emtricitabine/tenofovir disoproxil fumarate [TDF]), Biktarvy (bictegravir/ emtricitabine/tenofovir alafenamide [TAF]), Complera (emtricitabine/ rilpivirine/TDF), Delstrigo (doravirine/ lamivudine/TDF), Dovato (dolutegravir/ lamivudine), Genvoya (elvitegravir/ cobicistat/ emtricitabine/ TAF), Juluca (dolutegravir/ rilpivirine), Odefsey (emtricitabine, rilpivirine/TAF), Stribild (elvitegravir/ cobicistat/ emtricitabine/ TDF), Symfi, Symfi Lo (efavirenz/ lamivudine/TDF), Symtuza (darunavir/ cobicistat/emtricitabine/ TAF), Triumeq (abacavir/ dolutegravir/lamivudine)	Cabotegravir and rilpivirine will provide the first once monthly IM administered complete regimen option for HIV maintenance therapy. Injections will be required to be administered by a health care provider.  <i>Anticipated impact: Replacement spend; likely medical benefit</i>

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Multiple Sclerosis (MS)	Vumerity (diroximel fumarate) oral  Biogen/Alkermes	The treatment of relapsing forms of MS in adults	Pending FDA approval 10/17/2019	MS is an autoimmune disorder affecting the nerves of the brain and spinal cord. The protective nerve covering is damaged, leading to a variety of symptoms that can include vision changes, numbness, vertigo, bladder and bowel symptoms, weakness, muscle spasms, and eventually profound disability. MS affects nearly 1 million people in the U.S. The condition is mostly diagnosed between the ages of 20 and 50 and is more common in women. <sup>8</sup>  Relapsing MS is the most common form of the disease, affecting about 85% of patients, and is characterized by attacks (relapses) that are followed by periods of recovery (remissions). <sup>9</sup>	<b>Injectable/Infused Agents:</b> Avonex, Rebif (interferon beta-1a), Betaseron, Extavia (interferon beta-1b), glatiramer (e.g., Copaxone), Lemtrada (alemtuzumab), Ocrevus (ocrelizumab), Plegridy (peginterferon beta 1a), Tysabri (natalizumab)  <b>Oral Agents:</b> Aubagio (teriflunomide), Gilenya (fingolimod), Mavenclad (cladribine), Mayzent (siponimod), Tecfidera (dimethyl fumarate)	Vumerity is in the same drug class as Tecfidera and has a lower rate of gastrointestinal adverse events. It will be included in Specialty Guideline Management.  <i>Anticipated impact: Replacement spend</i>
	ozanimod oral  Celgene/Receptos		Pending FDA approval 03/25/2020			Ozanimod is in the same drug class as Gilenya and Mayzent and will provide an additional treatment option for patients with relapsing forms of MS. It will be included in Specialty Guideline Management.  <i>Anticipated impact: Replacement spend</i>

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Oral Oncology	avapritinib oral  Blueprint Medicines	The treatment of PDGFR $\alpha$ exon 18 mutant gastrointestinal stromal tumors (GIST), regardless of prior therapy, and fourth-line GIST, in adult patients	Pending FDA approval 02/14/2020	GIST is a type of cancer that originates in the gastrointestinal tract. Signs and symptoms of GIST can include blood in the stool or vomit, severe abdominal pain, severe fatigue, trouble or pain when swallowing and feeling full after only small amounts of food consumption.  There are 5,000 to 6,000 new cases of GIST diagnosed per year in the U.S. The median age of diagnosis is 60 and equally affects both genders. <sup>10</sup> PDGFR $\alpha$ mutations are found in 5-10% of GIST tumors. <sup>11</sup>	Gleevec (imatinib), Stivarga (regorafenib), Sutent (sunitinib)	Avapritinib has been granted Breakthrough Therapy designation for GIST and will be the first mutation targeted therapy approved for treatment. It will be included in Specialty Guideline Management.  <i>Anticipated impact: Replacement spend</i>
	zanubrutinib oral  BeiGene	The treatment of relapsed or refractory mantle cell lymphoma (MCL)	Pending FDA approval 02/27/2020	Lymphoma is the most common type of blood cancer and is divided into two major categories, Hodgkin lymphoma and non-Hodgkin lymphoma (NHL). MCL is one of about 70 different subtypes of NHL and accounts for roughly 6% of all NHL cases in the U.S. <sup>12</sup> MCL most often affects men over the age of 60. <sup>13</sup>  Most patients with MCL have disease involving multiple enlarged lymph nodes as well as involvement at other sites of the body, including the spleen, bone marrow, liver, or the gastrointestinal tract. Symptoms of MCL commonly include loss of appetite, weight loss, fever, night sweats, nausea and/or vomiting, abdominal pain, and discomfort as a result of an enlarged spleen or liver. <sup>12</sup>	<b>Injectable/Infused Agents:</b> Velcade (bortezomib)  <b>Oral Agents:</b> Calquence (acalabrutinib), Imbruvica (ibrutinib), Revlimid (lenalidomide)  Other IV and oral agents are used off-label for MCL	Zanubrutinib has been granted Breakthrough Therapy designation and will provide an additional treatment option for relapsed or refractory MCL. It will be included in Specialty Guideline Management.  <i>Anticipated impact: Replacement spend</i>

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- <sup>1</sup> RxPipeline, October 2019.
  - <sup>2</sup> Genetics Home Reference. Available at <https://ghr.nlm.nih.gov/condition/beta-thalassemia>. Accessed October 2, 2019.
  - <sup>3</sup> Bluebird bio Investor Presentation. Available at <http://investor.bluebirdbio.com/static-files/49bca517-85ca-4b4c-8f0f-26bebc3a5e0c>. Accessed October 4, 2019.
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  - <sup>6</sup> Centers for Disease Control and Prevention – HIV Basics. Available at <https://www.cdc.gov/hiv/basics/whatishiv.html>. Accessed October 1, 2019.
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  - <sup>8</sup> National Multiple Sclerosis Society. Available at <https://www.nationalmssociety.org/What-is-MS/MS-FAQ-s>. Accessed October 2, 2019.
  - <sup>9</sup> National Multiple Sclerosis Society. Available at <https://www.nationalmssociety.org/What-is-MS/Types-of-MS>. Accessed October 2, 2019.
  - <sup>10</sup> World Health Organization. GIST. Available at [https://www.who.int/selection\\_medicines/committees/expert/20/applications/GastrointestinalStromalTumour.pdf?ua=1](https://www.who.int/selection_medicines/committees/expert/20/applications/GastrointestinalStromalTumour.pdf?ua=1). Accessed October 1, 2019.
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  - <sup>12</sup> Leukemia & Lymphoma Society. Available at [https://www.lls.org/sites/default/files/file\\_assets/mantlecelllymphoma.pdf](https://www.lls.org/sites/default/files/file_assets/mantlecelllymphoma.pdf). Accessed October 2, 2019.
  - <sup>13</sup> Lymphoma Research Foundation. Available at <https://www.lymphoma.org/aboutlymphoma/nhl/mcl/>. Accessed October 2, 2019.

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