

Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q3 2019 – Q4 2019



Therapeutic Category	Product Name, Route of Administration and Manufacturer ¹	Proposed Indication ¹	Phase of Study ¹	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. ² Symptomatic cases of beta-thalassemia are estimated to occur in approximately 1 in 100,000 individuals in the general population. ³	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>
Multiple Sclerosis (MS)	Vumerity (diroximel fumarate) oral Biogen/ Alkermes	The treatment of relapsing-remitting MS	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. ⁴ 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). ⁵	Injectable/Infused Agents: Avonex, Rebif (interferon beta-1a); Betaseron, Extavia (interferon beta-1b); Copaxone (glatiramer) , Lemtrada (alemtuzumab), Ocrevus (ocrelizumab), Plegridy (peginterferon beta 1a), Tysabri (natalizumab) Oral Agents: Aubagio (teriflunomide), Gilenya (fingolimod), Mavenclad (cladribine), Mayzent (siponimod), Tecfidera (dimethyl fumarate)	Vumerity is in the same drug class as Tecfidera and has a low rate of gastrointestinal adverse events. It will be included in Specialty Guideline Management. <i>Anticipated impact: replacement spend</i>

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Muscular Dystrophy	golodirsen IV infusion Sarepta Therapeutics	The treatment of Duchenne muscular dystrophy (DMD) in patients with mutations amenable to exon 53 skipping	Pending FDA approval 08/19/2019	DMD is a genetic disorder characterized by muscle wasting and weakness starting in early childhood with rapid worsening. Most affected children will be wheelchair-dependent by age 10 to 12 years. By the late teens, the heart muscle and muscles around the rib cage can also be affected leading to heart and lung failure. DMD affects approximately 1 in every 3,500 live male births and rarely affects females. ⁶ Approximately 8 percent of DMD patients may be amenable to exon 53 skipping. ⁷	Disease-Modifying Therapy: Exondys 51 (eteplirsen) – targets a different mutation Symptomatic Therapy: Emflaza (deflazacort)	Golodirsen will provide the first disease modifying agent for patients with DMD with exon 53 mutations. It will be included in Specialty Guideline Management. <i>Anticipated impact: Incremental spend; likely medical benefit</i>
Oral Oncology	darolutamide oral Bayer HealthCare/ Orion Pharma	The treatment of high-risk, non-metastatic, castration-resistant prostate cancer (CRPC)	Pending FDA approval 10/27/2019	It is estimated that 1 in 9 men in the United States will develop prostate cancer in his lifetime. Prostate cancer is the second leading cause of cancer-related death for men. ⁸ Nearly all cases of prostate cancer will eventually become castration-resistant (i.e., resistant to androgen deprivation therapy, also called hormonal therapy). ⁹	Erleada (apalutamide), Xtandi (enzalutamide)	Darolutamide will provide an additional treatment option for men with non-metastatic CRPC. It will be included in Specialty Guideline Management. <i>Anticipated impact: Replacement spend</i>

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Oral Oncology (continued)	entrectinib oral Roche/ Genentech/ Ignyta	The treatment of advanced ROS1 fusion-positive non-small cell lung cancer (NSCLC), including in patients with brain metastases, and the treatment of NTRK fusion-positive advanced cancer, regardless of tissue origin	Pending FDA approval 08/18/2019	In the United States, lung cancer is the second most common cancer overall. NSCLC is the most common type of lung cancer accounting for nearly 84% of cases. ¹⁰ ROS1 alterations occur in approximately 1% of NSCLC patients. ¹¹ NTRK gene fusion positive cancer is rare. The prevalence is higher in rare pediatric and adult carcinomas; meanwhile, detection is lower in common tumors such as NSCLC, breast, colorectal, renal cell carcinoma, melanoma, and head and neck squamous cell. ^{12,13,14}	ROS1 fusion positive NSCLC: Xalkori (crizotinib); Off-label: Lorbrena (lorlatinib), Zykadia (ceritinib) NTRK fusion positive cancer: Vitrakvi (larotrectinib)	Entrectinib has been granted Breakthrough Therapy designation and will provide another oral option for ROS1 fusion positive NSCLC in patients with brain metastases and for the treatment of NTRK fusion positive advanced cancer. Entrectinib will be included in Specialty Guideline Management. <i>Anticipated impact: Replacement spend</i>
	fedratinib oral Celgene/ Impact Biomedicines	The treatment of myelofibrosis (MF)	Pending FDA approval 09/03/2019	MF is a chronic blood cancer. MF occurs when there is excessive scar tissue formation in the bone marrow which results in abnormal production of normal blood cells. Symptoms of MF include tiredness, weakness, or shortness of breath, fullness or discomfort or pain in the left upper area of the abdomen, fever, night sweats, weight loss, bone pain, easy bleeding, and susceptibility to infection. ¹⁵ MF affects fewer than 200,000 people in the US. ¹⁶ The annual incidence ranges from 0.3 to 1.5 cases per 100,000 persons. ¹⁷	Jakafi (ruxolitinib)	Fedratinib will provide a once daily option for patients with MF including in those who have insufficient response or cannot tolerate Jakafi. Fedratinib will be included in Specialty Guideline Management. <i>Anticipated impact: Incremental spend</i>

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Oral Oncology (continued)	Xpovio (selinexor) oral Karyopharm Therapeutics	The treatment of relapsed or refractory multiple myeloma (MM) in adults who have received at least 4 prior therapies, in combination with dexamethasone	Approved 07/03/2019	MM is a cancer of plasma cells. The overgrowth of plasma cells in the bone marrow can lead to low blood counts (red blood cells, white blood cells and platelets). Impaired ability to fight infection as well as bone fractures are other common features of MM. ¹⁸ The average lifetime risk for MM is approximately 0.8% with diagnosis most commonly in individuals 55 years of age and older. ¹⁹	Oral Agents: Farydak (panobinostat), Ninlaro (ixazomib), Pomalyst (pomalidomide), Revlimid (lenalidomide), Thalomid (thalidomide) Infused: Darzalex (daratumumab), Empliciti (elotuzumab), Kyprolis (carfilzomib), Velcade (bortezomib)	Selinexor provides a new mechanism of action and therapeutic option for patients who have not responded to multiple prior MM treatments. Selinexor will be included in Specialty Guideline Management. <i>Anticipated impact: Replacement spend</i>
Osteoporosis	teriparatide SC injection Alvogen/ Pfenex	The treatment of postmenopausal osteoporosis, primary or hypogonadal osteoporosis in men, and glucocorticoid-induced osteoporosis in men and women at high risk of fracture	Pending FDA approval 10/07/2019	Osteoporosis causes weakening and thinning of bone leading to an increased risk of fractures. It is estimated that 4.5 million women and 800,000 men over the age of 50 years have osteoporosis. One in two women and one in six men 50 years and older will suffer an osteoporosis-related fracture at some point in their lives. ²⁰ Corticosteroids can cause various adverse events including bone loss and fractures. It is estimated that 1% of the US population receives long-term glucocorticoid therapy. Of these patients, more than 10% will be diagnosed with a fracture. ²¹	Infused/Injectable Agents: Evenity (romosozumab), Forteo (teriparatide), ibandronate (e.g., Boniva), Miacalcin (calcitonin), Prolia (denosumab), Tymlos (abaloparatide), zoledronic acid (e.g., Reclast) Multiple oral agents are also approved for the prevention or treatment of osteoporosis.	Teriparatide is a therapeutic equivalent of Forteo. It will be included in Specialty Guideline Management. <i>Anticipated impact: Replacement spend</i>

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Rheumatoid Arthritis (RA)	upadacitinib oral AbbVie	The treatment of moderate-to-severe RA in adults	Pending FDA approval 08/20/2019	RA is chronic autoimmune and inflammatory disease which mainly attacks the joints in the hands, wrists, and knees. ²² RA affects more than 1.3 million Americans. RA typically begins between the ages of 30 and 50 years. About 75% of RA patients are women. ²³	Oral Agents: Olumiant (baricitinib), Xeljanz (tofacitinib), Xeljanz XR (tofacitinib) Disease-modifying antirheumatic drugs: Multiple oral and injectable formulations approved for moderate-to-severe RA	Upadacitinib, if approved, would provide another oral, once daily administered option for treatment of moderate-to-severe RA in adults. Upadacitinib will be included in Specialty Guideline Management. <i>Anticipated impact: Replacement spend</i>

¹ RxPipeline, July 2019

² Genetics Home Reference. Available at <https://ghr.nlm.nih.gov/condition/beta-thalassemia>. Accessed July 1, 2019.

³ National Organization for Rare Disorders. Available at <https://rarediseases.org/rare-diseases/thalassemia-major/>. Accessed July 1, 2019.

⁴ National Multiple Sclerosis Society. Available at: <https://www.nationalmssociety.org/What-is-MS/MS-FAQ-s>. Accessed June 27, 2019.

⁵ National Multiple Sclerosis Society. Available at: <https://www.nationalmssociety.org/What-is-MS/Types-of-MS>. Accessed June 27, 2019.

⁶ National Organization for Rare Disorders. Available at <https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/>. Accessed July 1, 2019.

⁷ Sarepta Therapeutics media release. Available at <https://www.globenewswire.com/news-release/2018/12/20/1670350/0/en/Sarepta-Therapeutics-Completes-Submission-of-New-Drug-Application-Seeking-Approval-of-Golodirsen-SRP-4053-in-Patients-with-Duchenne-Muscular-Dystrophy-Amenable-to-Skipping-Exon-53.html>. Accessed July 8, 2019.

⁸ American Cancer Society. Available at: <https://www.cancer.org/cancer/prostate-cancer/about/key-statistics.html>. Accessed July 1, 2019.

⁹ Luo J, Beer TM, Graff JN. Treatment of nonmetastatic castration-resistant prostate cancer. Available at <https://www.cancernetwork.com/oncology-journal/treatment-nonmetastatic-castration-resistant-prostate-cancer>. Accessed July 1, 2019.

¹⁰ American Society of Clinical Oncology. <https://www.cancer.net/cancer-types/lung-cancer-non-small-cell/statistics>. Accessed March 22, 2019.

¹¹ Gainor J, et al. Novel Targets in Non-Small Cell Lung Cancer: ROS and RET fusions. <http://theoncologist.alphamedpress.org/content/18/7/865.full.pdf+html>. Oncologist 2013;18:865-875. Accessed on March 22, 2019.

¹² Okamura R, et al. Analysis of NTRK Alterations in Pan-Cancer Adult and Pediatric Malignancies: Implications for NTRK-Targeted Therapeutics. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6329466/pdf/nihms-999212.pdf>. Accessed on March 27, 2019.

¹³ Cocco E, et al. NTRK fusion-positive cancers and TRK inhibitor therapy. Nature Reviews Clinical Oncology. 2018; 15: (12): 731-47. <https://www.nature.com/articles/s41571-018-0113-0>. Accessed on March 27, 2019.

¹⁴ Chen Y, Chi P. Basket trial of TRK inhibitors demonstrates efficacy in TRK fusion-positive cancers. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5992878/pdf/13045_2018_Article_622.pdf. Accessed on March 27, 2019.

¹⁵ Myeloproliferative Neoplasm Research Foundation. <http://www.mpnresearchfoundation.org/Primary-Myelofibrosis>. Accessed on March 22, 2019.

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- ¹⁶ Leukemia and Lymphoma Society. <https://www.lls.org/myeloproliferative-neoplasms/myelofibrosis>. Accessed on March 22, 2019.
- ¹⁷ National Institute of Health. <https://rarediseases.info.nih.gov/diseases/8618/myelofibrosis>. Accessed on March 22, 2019.
- ¹⁸ American Cancer Society. About Multiple Myeloma. Available at <https://www.cancer.org/cancer/multiple-myeloma/about/what-is-multiple-myeloma.html>. Accessed March 25, 2019.
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- ²⁰ American College of Rheumatology. Available at <https://www.rheumatology.org/I-Am-A/Patient-Caregiver/Diseases-Conditions/Osteoporosis>. Accessed July 1, 2019.
- ²¹ Buckley L, Guyatt G, Fink HA, et al. 2017 American College of Rheumatology Guideline for the Prevention and Treatment of Glucocorticoid-Induced Osteoporosis. Available at <https://www.rheumatology.org/Portals/0/Files/Guideline-for-the-Prevention-and-Treatment-of-GIOP.pdf>. Accessed July 1, 2019.
- ²² Centers for Disease Control and Prevention. Available at: <https://www.cdc.gov/arthritis/basics/rheumatoid-arthritis.html>. Accessed March 22, 2019.
- ²³ The American College of Rheumatology. <https://www.rheumatology.org/I-Am-A/Patient-Caregiver/Diseases-Conditions/Rheumatoid-Arthritis>. Accessed March 22, 2019.

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