Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q1 2019 – Q2 2019

CVSHealth

Therapeutic Category	Product Name, Route of Administration and Manufacturer[1]	Proposed Indication[1]	Phase of Study[1]	Disease Prevalence and Background	Select Available FDA-Approved Therapies	Comments
Multiple Sclerosis (MS)	Mayzent (siponimod) oral Novartis	The treatment of secondary progressive multiple sclerosis (SPMS)	Pending U.S. Food and Drug Administration (FDA) approval 03/25/2019	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 to profound disability.[2] MS affects approximately 400,000 people in the U.S. The condition is mostly diagnosed between the ages of 15 and 50 and is more common in women.[2] SPMS is characterized by progressive worsening of neurologic functions without attacks (relapses) and recovery (remissions). Within 10 and 25 years of initial MS diagnosis, about 50% and 90% of MS patients, respectively, will convert to SPMS.[3]	Novantrone (mitoxantrone) IV approved but seldom used due to toxicity. (Disease modifying treatments for relapsing MS are used off-label.)	If approved, Mayzent will be the first oral treatment option for delaying disability progression in SPMS patients. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Neuromuscular Disorders	Firdapse (amifampridine) oral Catalyst Pharmaceuticals	The treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults	Approved 11/28/2018	LEMS is a disease in which the immune system attacks the body's own tissues. The attack occurs at the connection between nerve and muscle cells which interferes with the ability to send chemical signals to trigger muscle contraction. This results in abnormal muscle contraction causing muscle weakness.[4] There are approximately 400 known cases of LEMS in the United States. Approximately 60 percent of LEMS cases are associated with small cell lung cancer and the onset of LEMS symptoms often precedes the detection of the cancer. LEMS patients with cancer tend to be older males with a long history of smoking.[5]	None (Cholinesterase inhibitors [e.g., pyridostigmine] and IV immunoglobulin therapy are used off-label for LEMS)	Firdapse was granted Breakthrough Therapy designation and represents the first FDA approved treatment option for LEMS. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Ophthalmic Disorders	Oxervate (cenegermin-bkbj) ocular solution Dompe Farmaceutici	The treatment of neurotrophic keratitis	Approved 08/22/2018	Neurotrophic keratitis is a rare, degenerative eye disease resulting from a loss of sensation in the cornea (the outermost layer of the eye). The loss of sensation impairs corneal health causing progressive damage to the top layer of the cornea, including corneal thinning as well as tearing and punctures in severe cases. The prevalence of neurotrophic keratitis has been estimated to be less than five in 10,000 individuals.[6]	None	Oxervate was granted Breakthrough Therapy designation and represents the first drug approved by the FDA for treatment of neurotrophic keratitis. *Anticipated impact: New spend*

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Oral Oncology	erdafitinib oral Janssen Pharmaceuticals	The treatment of locally advanced or metastatic urothelial cancer and certain fibroblast growth factor receptor (FGFR) genetic alterations whose tumors have progressed after prior chemotherapy	Pending FDA approval 05/18/2019	Urothelial cancer is the most common type of bladder cancer that starts in the interior lining of the bladder. It is the sixth most common type of cancer in the U.S. with an estimated 81,190 new cases diagnosed in the U.S. in 2018.[7] FGFR alterations occur in 10-20% of cases of metastatic urothelial cancer, and are associated with poor outcomes.[8]	None IV therapies FDA approved for urothelial cancer but not specifically for patients with FGFR alterations include: Bavencio (avelumab), Imfinzi (durvalumab), Keytruda (pembrolizumab), Opdivo (nivolumab), Tecentriq (atezolizumab)	Erdafitinib has been granted Breakthrough Therapy designation. If approved, it would be the first oral, targeted agent for urothelial cancer in patients with FGFR alterations who have failed other therapies. Erdafitinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical to pharmacy benefit)*
	quizartinib oral Daiichi Sankyo	The treatment of relapsed or refractory acute myeloid leukemia (AML) in patients with FLT3-ITD mutation	Pending FDA approval 05/25/2019	AML is a type of blood cancer that starts in certain immature blood cells and progresses quickly.[9] The average lifetime risk for AML is less than 0.5%. It occurs most commonly in individuals 45 years of age and older. Approximately 30% of patients diagnosed with AML have a FLT3-ITD mutation, which is associated with poor outcomes.[10]	Xospata (gilteritinib), (Traditional IV chemotherapy used off-label)	Quizartinib has been granted Breakthrough Therapy designation. Upon approval, it would provide another oral, targeted treatment option for FLT3-ITD mutated AML. Quizartinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical to pharmacy benefit)*

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Oral Oncology (continued)	selinexor Karyopharm Therapeutics	The treatment of penta-refractory multiple myeloma (MM), in combination with dexamethasone	Pending FDA approval 04/06/2019	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.[11] The average lifetime risk for MM is approximately 0.8% with diagnosis most commonly in individuals 55 years of age and older.[12]	**Oral Agents**: Farydak (panobinostat), Ninlaro (ixazomib), Pomalyst (pomalidomide), Revlimid (lenalidomide), Thalomid (thalidomide) **Infused:** Darzalex (daratumumab), Empliciti (elotuzumab), Kyprolis (carfilzomib), Velcade (bortezomib)	If approved, selinexor would represent a new mechanism of action and therapy option for patients that have not responded to multiple prior MM treatments. Selinexor will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Postpartum Depression (PPD)	Zulresso (brexanolone) IV infusion Sage Therapeutics	The treatment of moderate-to-severe postpartum depression	Pending FDA approval 03/19/2019	PPD is a serious mood disorder that can last for many weeks or months after delivering a baby.[13] Some women with moderate-to-severe PPD may experience suicidal ideation or obsessive thoughts of harming their infants. Up to 1 in 7 women experiences PPD. About 25-50% of women will have a recurrence after a subsequent pregnancy.[14]	None (Traditional oral antidepressant medications used off-label)	Zulresso (brexanolone) has been granted Breakthrough Therapy designation. If approved, it would be the first drug indicated for PPD and would offer a highly effective, rapidly acting treatment. *Anticipated impact: Incremental spend; primarily medical benefit*

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Psoriasis	risankizumab subcutaneous injection AbbVie/ Boehringer Ingelheim	The treatment of moderate-to-severe chronic plaque psoriasis in adults	Pending FDA approval 04/25/2019	Psoriasis is an immune disease primarily affecting the skin and joints. The most common form, plaque psoriasis, causes thick, scaly patches on the skin that often can itch, cause pain, crack and bleed.[15] Psoriasis is estimated to affect 7.5 million Americans, or about 2.2% of the population[16], with the plaque psoriasis subtype accounting for 80-90% of cases.[17]	**Topical Agents:** Various creams and ointments used for mild-to-moderate psoriasis **Oral Agents:** Otezla (apremilast) **SC injectable biologic agents:** Cimzia (certolizumab pegol), Cosentyx (secukinumab), Enbrel (etanercept), Humira (adalimumab), Ilumya (tildrakizumab), Siliq (brodalumab), Stelara (ustekinumab), Taltz (ixekizumab), Tremfya (guselkumab)	Risankizumab, if approved, would provide another subcutaneously administered option for treatment of plaque psoriasis. Risankizumab will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Seizure Disorders	Diacomit (stiripentol) oral Biocodex	The treatment of seizures associated with Dravet syndrome in patients 2 years of age and older taking clobazam	Approved 08/20/2018	Dravet syndrome is a rare, severe, lifelong form of epilepsy that begins in the first year of life with frequent and prolonged seizures. While seizures persist, other comorbidities, such as developmental delay, are often not evident until the second or third year of life.[18] Dravet syndrome affects an estimated 1 in 15,700 individuals in the U.S., or 0.0064% of the population.[19]	Epidiolex (cannabadiol) (Traditional anti-seizure medications used off-label)	Diacomit represents an additional treatment option for Dravet syndrome. *Anticipated impact: Incremental spend*

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