Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q2 2020 – Q3 2020

CVSHealth

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
Multiple Sclerosis (MS)	Bafiertam (monomethyl fumarate) oral Banner Life Sciences	The treatment of relapsing forms of MS in adults	Tentative approval; 11/16/2018 Full approval expected following expiration of Tecfidera patents 06/20/2020	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 United States. The condition is mostly diagnosed between the ages of 20 and 50 and is more common in women.[2] 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).[3]	**Injectable/Infused Agents:** Avonex, Rebif (interferon beta-1a), Betaseron, Extavia (interferon beta-1b), glatiramer (e.g., Copaxone), Lemtrada (alemtuzumab), Ocrevus (ocrelizumab), Plegridy (peginterferon beta 1a), Tysabri (natalizumab) **Oral Agents:** Aubagio (teriflunomide), Gilenya (fingolimod), Mavenclad (cladribine), Mayzent (siponimod), Tecfidera (dimethyl fumarate), Vumerity (diroximel fumarate)	Bafiertam is bioequivalent to the active ingredient of Tecfidera, and will provide an alternative treatment option for patients with relapsing forms of MS. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
	ofatumumab SC injection Genmab/Novartis		Pending FDA approval 06/24/2020			Ofatumumab is a monthly, SC, self-administered injection which is in the same drug class as infused Ocrevus. Ofatumumab will provide an additional treatment option for relapsing forms of MS. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
	Zeposia (ozanimod) oral Celgene/ Receptos		Approved 03/25/2020			Zeposia 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. *Anticipated impact: Replacement spend*

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Muscular Dystrophy	viltolarsen IV NS Pharma	The treatment of Duchenne muscular dystrophy (DMD) in patients with mutations amenable to exon 53 skipping	Pending FDA approval 06/02/2020	DMD is a rare, genetic muscle disorder due to the alterations of a protein called dystrophin which helps keep muscle cells intact. DMD is characterized by progressive muscle weakness and wasting. Symptoms of DMD occurs in early childhood usually between the ages of 2 and 3. In the early stages of DMD, affected individuals will have difficulty jumping, running, walking, and maintaining balance. By their teenage years, most individuals will require a wheelchair. As the disease progresses, the heart and respiratory muscles will be affected.[4] DMD primarily affects boys, but in rare cases can affect girls. The prevalence of DMD is approximately one in every 3,500 live male births.[5] Approximately 8% of DMD patients may be amenable to exon 53 skipping.[6]	**Disease-Modifying Therapy:** Vyondys 53 (golodirsen) Exondys 51 (eteplirsen] – targets a different mutation **Symptomatic Therapy:** Emflaza (deflazacort)	Viltolarsen will provide an additional treatment option for individuals with DMD amenable to exon 53 mutations. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend; likely medical benefit*
Neuromuscular	risdiplam oral Genentech/ Roche/PTC Therapeutics	The treatment of types 1, 2 and 3 spinal muscular atrophy (SMA)	Pending FDA approval 08/24/2020	SMA is a rare, genetic disease caused by inadequate production of the survival motor neuron (SMN) protein. It is characterized by muscle weakness and wasting primarily in infants and children, though adults may be affected. [7] SMA affects one in 6,000 to one in 10,000 live births. There are five types of SMA, which are based on the severity of the disorder and the age of symptom onset. However, types 1, 2 and 3 account for over 95% of cases and typically have an onset between infancy and early childhood.[8]	**Disease-modifying therapy**: Spinraza (nusinersen) intrathecal injection (chronic therapy) **Gene therapy**: Zolgensma (onasemnogene abeparvovec-xioi) one-time IV infusion	Risdiplam was granted Breakthrough Therapy designation. It is in the same drug class as Spinraza, and will provide the first oral treatment option for SMA. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical benefit)*

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Oral Oncology	capmatinib oral Novartis	The treatment of metastatic MET exon 14 skipping-mutated non-small cell lung cancer (NSCLC)	Pending FDA approval 08/10/2020	In the U.S., lung cancer is the second most common cancer and the leading cause of cancer death among men and women. Close to 540,000 people are living with lung cancer.[9] NSCLC is the most common type of lung cancer accounting for 84% of all cases.[10] MET exon 14 skipping occurs in approximately 3 to 4% of all newly diagnosed individuals with NSCLC.[11] These individuals tend to be older and have a poor prognosis.[12]	Xalkori (crizotinib); used off-label	Capmatinib was granted Breakthrough Therapy designation. If approved, capmatinib will be the first FDA approved treatment for MET exon 14 skipping-mutated NSCLC. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
	cedazuridine/ decitabine oral Astex Pharmaceuticals/ Otsuka America Pharmaceutical/ Taiho Pharmaceutical	The treatment of previously untreated intermediate and high-risk myelodysplastic syndrome (MDS), including chronic myelomonocytic leukemia (CMML), in adults	Pending FDA approval 08/12/2020	MDS and CMML occurs when the bone marrow does not function normally leading to abnormal development of blood cells. In one-third of patients, MDS and CMML will progress to acute myeloid leukemia, a rapidly growing cancer of bone marrow cells.[13] Anemia, frequent infections, easy bruising and bleeding are the most common symptoms of MDS. In the U.S., the prevalence of MDS is estimated to be from 60,000 to 170,000 with an estimated 20,000 new patients diagnosed each year. MDS can occur in any age group, but most cases are found in older adults typically in their 70s.[14] Approximately 66% and 12.8% of MDS cases are intermediate to high-risk, respectively.[15] The incidence of CMML in the U.S. is approximately 1,100 new cases per year. CMML occurs more commonly in men than women and most cases are diagnosed in people 60 years and older.[16]	Multiple agents are approved or used off label for MDS or CMML based on risk classification and eligibility for stem cell transplant including Dacogen (decitabine) IV and Vidaza (azacitidine) IV, SQ.	Cedazuridine/decitabine is in the same drug class as IV decitabine and IV/SC azacitidine and will provide an oral alternative for these agents for the treatment of MDS and CMML. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical benefit)*

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Oral Oncology (continued)	selpercatinib oral Eli Lilly/ Loxo Oncology	The treatment of metastatic RET fusion-positive NSCLC in patients who have progressed following chemotherapy and an anti-PD-1/PD-L1 therapy	Pending FDA approval 07/29/2020	In the U.S., 540,000 people are living with lung cancer. NSCLC is the most common type of lung cancer accounting for 84% of all cases. An estimated 2% of all lung cancer tumors harbor RET fusions.[17]	**Off-label use:** Cabometyx/Cometriq (cabozantinib), Caprelsa (vandetanib)	Selpercatinib was granted Breakthrough Therapy designation and will be the first FDA approved agent targeting RET mutations for NSCLC, thyroid cancer, and MTC. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
		The treatment of relapsed or refractory RET-mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancer in patients without an acceptable alternative treatment option	Pending FDA approval 07/29/2020	Thyroid cancer is relatively uncommon compared to other cancers, occurring more in women than men. Diagnoses of thyroid cancer occurs at a younger age than most adult cancers.[18] In the U.S., close to 630,000 individuals have thyroid cancer with approximately 53,000 new patients diagnosed yearly. MTC accounts for approximately 2% of all cases.[19] RET fusions have been identified in 10 to 20% of thyroid cancers and RET mutations occur in approximately 60% of MTC cases.[20]	**Approved for thyroid cancer, but not specific for RET fusions:** Caprelsa (vandetanib), Cometriq (cabozantinib), Lenvima (lenvatinib), Nexavar (sorafenib), traditional IV chemotherapy **Off-label use:** Cabometyx (cabozantinib), Sutent (sunitinib), Votrient (pazopanib)	

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Oral Oncology (continued)	Tukysa (tucatinib) oral Array Biopharma/ Pfizer/Seattle Genetics	The treatment of HER-2 positive, advanced, unresectable or metastatic breast cancer in combination with capecitabine and trastuzumab, in patients who have received at least one prior HER2-directed agent	Approved 04/17/2020	Breast cancer is the second most common cancer among women in the U.S.[21] Approximately 12% of women will develop breast cancer during their lifetime. Certain hormones (estrogen, progesterone) or proteins (HER2) can promote the growth of breast cancer.[22] Approximately 20% of breast cancers are HER-2 positive; in general, this type of breast cancer spreads more aggressively.	**HER-2 Targeted Agents:** **Oral Agents:** Nerlynx (neratinib), Tykerb (lapatinib) **IV Agents:** Enhertu (fam-trastuzumab deruxtecan-nxki), Herceptin (trastuzumab), Herceptin biosimilar products (e.g., Herzuma [trastuzumab-pkrb], Kanjinti [trastuzumab-anns], Ogivri [trastuzumab-dkst], Ontruzant [trastuzumab-dttb], Trazimera [trastuzumab-gyyb]), Herceptin Hylecta (trastuzumab/hyaluronidase-oysk), Kadcyla (ado-trastuzumab emtansine), Perjeta (pertuzumab)	Tukysa was granted Breakthrough Therapy designation. It provides an additional oral treatment option for HER-2 positive breast cancer. Tukysa will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend*

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Osteoporosis	Bonsity (teriparatide) SC injection Alvogen/Pfenex	The treatment of postmenopausal women with osteoporosis at high risk for fracture, to increase bone mass in men with primary or hypogonadal osteoporosis at high risk for fracture, and the treatment of men and women with osteoporosis associated with sustained systemic glucocorticoid therapy at high risk for fracture	Approved 10/04/2019	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 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.[23] Corticosteroids can cause various adverse events including bone loss and fractures. It is estimated that 1% of the U.S. population receives long-term glucocorticoid therapy. Of these patients, more than 10% will be diagnosed with a fracture.[24]	**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 in addition to calcitonin nasal are also approved for the prevention or treatment of osteoporosis.	Bonsity is a therapeutic equivalent of Forteo. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Rare Disorders — Other	inebilizumab IV Viela Bio	The treatment of neuromyelitis optica spectrum disorder (NMOSD) in adults	Pending FDA approval 06/11/2020	NMOSD is a chronic autoimmune disorder affecting the central nervous system that is characterized by inflammation of the optic nerve and spinal cord. Symptoms include eye pain, vision loss, sensory loss, bowel and bladder dysfunction, and paralysis/ impaired mobility. Patients typically experience repeated attacks with periods of remission in between though permanent blindness or impaired mobility is common in recurring cases. [25] The prevalence of NMOSD is estimated to be one to 10 per 100,000 individuals. Though it can be diagnosed at any age, middle-aged women are most commonly affected and are more likely to have the recurring form than men. Approximately 80% of patients with NMOSD test positive for the aquaporin-4 (AQP-4) antibody, which has been associated with increased disease severity.[25,26]	Soliris (eculizumab) IV infusion Approved for NMOSD in adults who are AQP4+	Both twice yearly inebilizumab and satralizumab were granted Breakthrough Therapy designation. These agents will provide alternative options for AQP4+ and AQP4- NMOSD as either monotherapy or add-on therapy to oral immunosuppression (e.g., azathioprine, corticosteroids, and/or mycophenolate mofetil). Satralizumab will provide the first self-administered SC option for NMOSD. Both agents will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend*
	satralizumab SC Genentech/ Roche	The treatment of NMOSD in adults and adolescents	Pending FDA approval 04/30/2020			
Rheumatoid Arthritis (RA)	filgotinib oral Galapagos/ Gilead	The treatment of moderate-to-severe RA	Pending FDA approval 08/19/2020	RA is a chronic autoimmune and inflammatory disease which mainly attacks the joints in the hands, wrists and knees.[27] RA affects more than 1.3 million Americans. RA typically begins between the ages of 30 and 50. About 75% of RA patients are women.[28]	**Oral JAK Inhibitors:** Olumiant (baricitinib), Rinvoq (upadacitinib), Xeljanz/Xeljanz XR (tofacitinib) **Other disease-modifying antirheumatic drugs:** Multiple oral and injectable products are approved for moderate-to-severe RA	Filgotinib is in the same drug class as Olumiant, Rinvoq, and Xeljanz and will provide an additional oral treatment option for RA. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Seizure Disorders	Cortrophin Gel (corticotrophin) IM and SC injection ANI Pharmaceutical	The treatment of infantile spasms, multiple sclerosis, rheumatic disorders, collagen disease, dermatologic diseases, allergic states, ophthalmic diseases, respiratory diseases, and edematous state	Pending FDA approval 07/24/2020	Infantile spasms is a severe epilepsy syndrome that typically presents within the first year of life.[29] The spasms tend to occur in multiple clusters and infants may have hundreds of seizures per day. The majority of infants develop severe developmental delays. Infantile spasms occurs in more than 1,200 infants yearly in the U.S.[30]	H.P. Acthar Gel (repository corticotropin)	Cortrophin Gel has the same active ingredient as H.P. Acthar Gel. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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