Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q3 2020 – Q4 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
Growth Hormone Deficiency (GHD)	somapacitan SC injection Novo Nordisk Pharmaceuticals	The treatment of GHD in adults	Pending FDA approval 09/01/2020	GHD is a rare disorder which is characterized by the insufficient secretion of growth hormone, an essential hormone which maintains normal body structure and metabolism.[2] Adult growth hormone deficiency (AGHD) can be present from birth (congenital), acquired later in life due to structural damage or trauma, or idiopathic (unknown). Signs and symptoms of AGHD may include increase in fat mass, decrease in muscle mass, fatigue, lipid abnormalities and depression.[3] The true prevalence of AGHD is uncertain, but is estimated to be 2-3 per 10,000 people.[4] Both males and females are affected equally.[3]	**SC, daily administered somatropin (recombinant human growth hormone) agents:** Genotropin, Humatrope, Norditropin, Nutropin AQ, Omnitrope, Saizen, Zomacton	Somapacitan is a once weekly, SC, self-administered growth hormone. Somapacitan will provide a less frequent administration schedule compared to currently available therapies. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Hereditary Angioedema (HAE)	berotralstat oral BioCryst Pharmaceuticals	The prevention of HAE attacks in adults and adolescents	Pending FDA approval 12/03/2020	HAE is a rare inherited recurrent disorder characterized by edema (swelling) of the skin or tissues surrounding the upper respiratory and gastrointestinal tracts.[5] HAE is self-limiting and typically resolves in 2 to 5 days without treatment. However, fatal asphyxiation (suffocation) could result due to the involvement of the respiratory tract. There is no known precipitating cause for most HAE attacks. Prevention treatments can reduce the number and severity of HAE acute attacks.[6] HAE is estimated to affect 1 in 50,000 people. Symptoms of HAE typically start in early childhood. HAE affects males and females equally.[5,6]	**HAE Attack Prevention Agents:** SC: Haegarda (C1 esterase inhibitor subcutaneous [human]), Takhzyro (lanadelumab-flyo) IV: Cinrzye (C1 esterase inhibitor [human])	Berotralstat will provide an oral option for prevention of HAE attacks. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Multiple Sclerosis (MS)	Bafiertam (monomethyl fumarate) oral Banner Life Sciences	The treatment of relapsing forms of MS in adults	Approved 04/28/2020	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.[7] 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).[8]	**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), Zeposia (ozanimod)	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 09/24/2020			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*

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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 08/07/2020	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 occur 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.[9] DMD primarily affects boys but in rare cases can affect girls. The prevalence of DMD is approximately 1 in every 3,500 live male births.[10] Approximately 8% of DMD patients may be amenable to exon 53 skipping.[11]	**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: Replacement 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.[12] SMA affects 1 in 6,000 to 1 in 10,000 live births. There are 5 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.[13]	**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	azacitidine oral Bristol-Myers Squibb/Celgene	The maintenance treatment of newly diagnosed acute myeloid leukemia (AML)	Pending FDA approval 09/03/2020	AML is a rapidly progressive type of blood and bone marrow cancer. Signs and symptoms of AML include: fever, bone pain, fatigue, shortness of breath, pale skin, frequent infections, easy bruising, and unusual bleeding such as nosebleeds and bleeding from the gums.[14] Annually, there are approximately 20,000 new diagnoses of AML. AML generally occurs in the elderly with the average age of diagnosis occurring at 68 years. AML is more common among men than women.[15]	**Off-label chemotherapy agent use:** Vidaza (azacitidine) IV, Dacogen (decitabine) IV	Azacitidine will be an oral option for maintenance treatment of AML. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
	Inqovi (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	Approved 07/07/2020	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.[16] Anemia, frequent infections, easy bruising and bleeding are the most common symptoms of MDS. In the U.S., 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.[17] Approximately 66% and 12.8% of MDS cases are intermediate to high-risk, respectively.[18] 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.[19]	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, Vidaza (azacitidine) IV, SC	Inqovi 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)	pralsetinib oral Blueprint Medicines	The treatment of RET fusion-positive non-small cell lung cancer (NSCLC)	Pending FDA approval 11/23/2020	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.[20,21] An estimated 2% of all lung cancer tumors harbor RET fusions.[22]	Retevmo (selpercatinib) **Off-label use:** Cabometyx/Cometriq (cabozantinib), Caprelsa (vandetanib)	Pralsetinib is an oral treatment option for RET positive NSCLC. Pralsetinib was granted Breakthrough Therapy designation. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Rare Disorders - Other	satralizumab SC Chugai Pharmaceutical/ Genentech/ Roche	The treatment of neuromyelitis optica spectrum disorder (NMOSD) in adults and adolescents	Pending FDA approval 08/15/2020	NMOSD is a chronic, ultra-rare, 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. [23] In the U.S., there is an estimated 10,000 people diagnosed with NMOSD. Of those diagnosed, approximately 8,000 are anti-aquaporin-4 (AQP4) antibody positive, for which that status has been associated with increased disease severity. Although NMOSD can be diagnosed at any age, middle-aged women are most commonly affected and are more likely to have the recurring form than men.[24]	Soliris (eculizumab) IV infusion, Uplizna (inebilizumab-cdon) IV infusion Both agents approved for NMOSD in adults who are AQP4 antibody positive	Satralizumab was granted Breakthrough Therapy designation and will provide the first self-administered SC option for NMOSD. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical benefit)*

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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 affects the joints in the hands, wrists and knees.[25] 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.[26]	**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*
Seizure Disorders	Cortrophin Gel (corticotrophin) IM and SC injection ANI Pharmaceuticals	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 2H 2020	Infantile spasms is a severe epilepsy syndrome that typically presents within the first year of life.[27] 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 occur in more than 1,200 infants yearly in the U.S.[28]	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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