Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q4 2020 – Q1 2021

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)	Sogroya (somapacitan-beco) subcutaneous (SC) injection Novo Nordisk Pharmaceuticals	The treatment of GHD in adults	Approved 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 cause). 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	Sogroya is a once weekly, SC, self-administered growth hormone. Sogroya 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)	Orladeyo (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 often 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 therapies 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: Cinryze (C1 esterase inhibitor [human]) **HAE Attack Treatment Agents:** SC: Firazyr (icatibant), Kalbitor (ecallantide) IV: Berinert (C1 esterase inhibitor [human]), Ruconest (C1 esterase inhibitor recombinant)	Orladeyo 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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Human Immuno-deficiency Virus (HIV)	Cabenuva (cabotegravir + rilpivirine) intramuscular (IM) injection GlaxoSmithKline/ Janssen Pharmaceuticals/ Johnson & Johnson/ Pfizer/ ViiV Healthcare	The maintenance treatment of HIV type-1 infection in adults without resistance to cabotegravir or rilpivirine	Pending FDA approval 01/01/2021	HIV is a virus which attacks the body's immune system, making individuals more vulnerable to other infections and diseases. HIV is treatable but not curable. HIV can lead to acquired immunodeficiency syndrome, if not treated. An estimated 1.2 million people are living with HIV in the United States and 14% of them are not aware they have the disease. Annually, approximately 38,000 people receive an HIV diagnosis.[7]	**Complete regimens:** Atripla (efavirenz/emtricitabine/ tenofovir disoproxil fumarate) Biktarvy (bictegravir/emtricitabine/ tenofovir alafenamide) Complera (emtricitabine/rilpivirine/ tenofovir disoproxil fumarate) Delstrigo (doravirine/lamivudine/ tenofovir disoproxil fumarate) Dovato (dolutegravir/lamivudine) Genvoya (elvitegravir/cobicistat/ emtricitabine/tenofovir alafenamide) Juluca (dolutegravir/rilpivirine) Odefsey (emtricitabine/rilpivirine/ tenofovir alafenamide) Stribild (elvitegravir/cobicistat/ emtricitabine/tenofovir disoproxil fumarate) Symfi/Symfi Lo (efavirenz/ lamivudine/tenofovir disoproxil fumarate) Symtuza (darunavir/cobicistat/ emtricitabine/tenofovir alafenamide) Triumeq (abacavir/dolutegravir/ lamivudine)	If approved, Cabenuva will be the first long-acting, complete regimen for the treatment of HIV-1 infection in adults without resistance to cabotegravir and rilpivirine. Cabenuva will be administered every month after a 4-week oral once daily lead in period. *Anticipated impact: Replacement spend, shift to medical spend*

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Lysosomal Storage Disorders	arimoclomol oral Orphazyme	The treatment of Niemann-Pick type C (NPC) disease	Pending FDA approval 03/17/2021	NPC is a rare, progressive lysosomal storage disorder which results in an abnormal accumulation of cholesterol and other fats in various tissues. This accumulation of lipids within the body causes damage to various organs including the liver, spleen, and brain, as well as loss of cognitive skills, seizures, and difficulty with speech, swallowing, and feeding.[8] The incidence of NPC is 1 in 100,000 live births. In the United States and Europe, there are approximately 1,100 diagnosed patients with NPC.[9]	There are no FDA approved treatments for NPC in the United States. Zavesca (miglustat) is approved in Europe for NPC and is approved in the United States for Gaucher disease.	Arimoclomol was granted Breakthrough Therapy designation and will be the first FDA approved agent for treatment of NPC disease. It will be included in Specialty Guideline Management. *Anticipated impact: New spend, pharmacy benefit*
	pegunigalsidase alfa intravenous (IV) Chiesi USA/ Protalix BioTherapeutics	The treatment of Fabry disease in adults	Pending FDA approval 01/27/2021	Fabry disease is a rare, inherited disorder that prevents the body from making alpha-galactosidase, which is needed to break down fatty substances. As a result of the accumulation of fatty substances, blood vessels are narrowed which affects the skin, kidney, heart, brain, and nervous system.[10] Life-threatening complications such as arrhythmias, myocardial infarction (heart attack), renal failure, and strokes can occur. Fabry disease affects an estimated 1 in 40,000 to 60,000 males. It also affects females but the incidence is unknown. Males are typically more severely affected than females.[11]	Fabrazyme (agalsidase beta) IV Galafold (migalastat) oral − limited to those with an amenable genetic variation	Pegunigalsidase alfa could provide an alternative treatment option for Fabry disease. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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Multiple Sclerosis (MS)	ponesimod oral Actelion/Johnson & Johnson	The treatment of relapsing forms of MS in adults	Pending FDA approval 03/18/2021	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.[12] 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).[13]	**Injectable/Infused Agents:** Avonex, Rebif (interferon beta-1a), Betaseron, Extavia (interferon beta-1b), glatiramer (e.g., Copaxone), Kesimpta (ofatumumab), Lemtrada (alemtuzumab), Ocrevus (ocrelizumab), Plegridy (peginterferon beta-1a), Tysabri (natalizumab) **Oral Agents:** Aubagio (teriflunomide), Bafiertam (monomethyl fumarate), dimethyl fumarate (generic equivalent of Tecfidera), Gilenya (fingolimod), Mavenclad (cladribine), Mayzent (siponimod), Tecfidera (dimethyl fumarate), Vumerity (diroximel fumarate), Zeposia **(ozanimod)**	Ponesimod is in the same drug class as Gilenya, Mayzent and Zeposia. Ponesimod will provide an additional oral 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	Amondys 45 (casimersen) IV Sarepta Therapeutics	The treatment of Duchenne muscular dystrophy (DMD) in patients with mutations amenable to exon 45 skipping	Pending FDA approval 02/25/2021	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.[14] 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.[15] Approximately 8% of DMD patients may be amenable to exon 45 skipping.[16]	**Disease-Modifying Therapy:** Exondys 51 (eteplirsen) IV, Viltepso 53 (viltolarsen) IV, Vyondys 53 (golodirsen) IV – each targets different mutations **Symptomatic Therapy:** Emflaza (deflazacort) oral	Amondys 45 will provide a treatment option for individuals with DMD amenable to exon 45 mutations. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, primarily medical benefit*
Oral Oncology	Oraxol (paclitaxel and encequidar) oral Athenex Pharmaceuticals	The treatment of metastatic breast cancer (BC) as monotherapy	Pending FDA approval 02/28/2021	BC is a common cancer in American women with a median age at diagnosis of 62 years. The average risk of a woman developing BC sometime in her life is about 13%. The U.S. prevalence of BC is approximately 3.5 million. An estimated 6% of cases are metastatic at the time of diagnosis.[17]	**Taxanes approved for BC:** Abraxane (paclitaxel protein-bound), docetaxel (e.g., Taxotere), paclitaxel (e.g., Taxol)	Oraxol is an oral alternative to IV paclitaxel and other taxanes for metastatic BC. It will be included in Specialty Guideline Management. *Anticipated spend: Replacement spend (shift to pharmacy benefit)*

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Oral Oncology (continued)	relugolix oral Myovant/ Sumitomo Dainippon Pharma	The treatment of androgen-sensitive, advanced prostate cancer (PC)	Pending FDA approval 12/20/2020	PC is the second most prevalent form of cancer in men with a median age of diagnosis of 66 years. PC is likely to occur in men with a family history of PC and of African American descent. The U.S. prevalence of PC is approximately 3.2 million. About 1 in 9 men will be diagnosed with PC during his lifetime.[18]	**Gonadotropin-releasing hormone (GnRH) receptor agonists:** leuprolide IM/SC (e.g., Eligard, Lupron Depot), Trelstar (triptorelin) IM, Vantas (histrelin) SC, Zoladex (goserelin) SC **GnRH antagonist:** Firmagon (degarelix) SC **First generation antiandrogen:** bicalutamide oral, flutamide oral, nilutamide oral, (generally used in combination with other agents)	Relugolix is a GnRH receptor antagonist that will provide an oral option for androgen-sensitive, advanced PC. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift to pharmacy benefit)*
	umbralisib oral TG Therapeutics	The treatment of marginal zone lymphoma (MZL) in adults who have received at least 1 prior anti-CD20 based regimen	Pending FDA approval 02/15/2021	MZL is a slow-growing B-cell non-Hodgkin lymphoma (NHL). MZL occurs slightly more in women than men. The average age at diagnosis is 60 years.[19] MZL accounts for approximately 8% of all NHL cases. In the United States, the annual incidence of newly diagnosed patients is estimated to be 7,500.[20] Approximately 15-30% of patients relapse and require rescue therapy.[21]	**FDA approved, NCCN supported therapies:** Imbruvica (ibrutinib) oral, Revlimid (lenalidomide) oral + rituximab IV (e.g., Rituxan, Truxima, Ruxience) **Select Off-label NCCN recommended therapies:** Aliqopa (copanlisib) IV, Copiktra (duvelisib) oral, Gazyva (obinutuzumab) IV + bendamustine IV, rituximab IV (e.g., Rituxan, Truxima, Ruxience), traditional chemotherapy, Zydelig (idelalisib) oral	Umbralisib will provide an additional oral treatment option for MZL in adults who have failed at least 1 prior anti-CD20 based regimen. Umbralisib was granted Breakthrough Therapy designation. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Oral Oncology (continued)	tepotinib oral EMD Serono/Merck KGaA	The treatment of non-small cell lung cancer (NSCLC) in patients with MET exon 14 skipping alterations	Pending FDA approval 02/25/2021	In the United States, lung cancer is the second most common cancer and leading cause of death among men and women. Close to 540,000 people are living with lung cancer. NSCLC is the most common type of lung cancer accounting for 84% of all cases.[22,23] MET exon 14 skipping occurs in approximately 3-4 % of all newly diagnosed individuals with NSCLC.[24] These individuals tend to be older and have a poor prognosis.	Tabrecta (capmatinib)	Tepotinib will provide an additional oral treatment option for NSCLC in patients with MET exon 14 skipping alterations. Tepotinib was granted Breakthrough Therapy designation. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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