Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q1 2021 – Q2 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
Atopic Dermatitis (AD)	abrocitinib oral Pfizer	The treatment of moderate-to-severe AD in patients aged 12 years and older	Pending FDA approval 04/27/2021	AD, also referred to as eczema, is a chronic inflammatory disorder affecting the skin. Common symptoms include widespread areas of dry skin, itching, and red rashes. Scratching may lead to oozing and crusting, as well as thickening and hardening of the skin. Skin infections may also occur. AD affects 10 to 20% of children and 5 to 10% of adults.[2] Approximately 40% of patients have moderate-to-severe disease.[3]	Dupixent (dupilumab) SC Approved oral agents seeking supplemental indications for AD: Olumiant (baricitinib) – pending FDA approval 03/15/2021, Rinvoq (upadacitinib) – pending FDA approval 04/19/2021 Numerous topical therapies may be used	Abrocitinib was granted Breakthrough Therapy designation and will provide an oral therapy option for moderate-to-severe AD. Tralokinumab will provide an additional self-injected therapy option. Both agents will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
	tralokinumab subcutaneous (SC) injection LEO Pharma	The treatment of moderate-to-severe AD in adults	Pending FDA approval 05/09/2021			
Growth Hormone Deficiency (GHD)	lonapegsomatropin SC injection Ascendis Pharmaceuticals	The treatment of GHD in pediatrics	Pending FDA approval 06/25/2021	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. Signs and symptoms of GHD may include slow growth, low blood sugar levels, poor development of bones in the middle of the face. GHD occurs in approximately 1 in every 3,800 infants.[4]	**SC, daily administered somatropin (recombinant human growth hormone) agents:** Genotropin, Humatrope, Norditropin, Nutropin AQ, Omnitrope, Saizen, Zomacton **Long-acting somatropin agent:** Sogroya (somapacitan-beco) – indicated for adult GHD	Lonapegsomatropin is an additional once weekly, SC, self-administered growth hormone product that will offer a less frequent administration schedule compared to currently available therapies for pediatric GHD. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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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	Approved 01/21/2021	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.[5]	**Oral 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)	Cabenuva is 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 benefit*

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Lysosomal Storage Disorders (LSDs)	arimoclomol oral Orphazyme	The treatment of Niemann-Pick type C (NPC) disease	Pending FDA approval 06/17/2021	NPC is a rare, progressive LSD 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.[6] 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.[7]	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*
	avalglucosidase alfa intravenous (IV) Genzyme/ Sanofi	The treatment of Pompe disease (glycogen storage disease type II)	Pending FDA approval 05/18/2021	Pompe disease is a rare, inherited LSD that leads to accumulation of glycogen, a complex sugar, in muscles as well as other organs and tissues. In the most severe type, infantile-onset Pompe disease, symptoms generally begin a few months after birth and include muscle weakness, decreased muscle tone, heart defects, failure to thrive, and breathing difficulties. If untreated, infantile-onset Pompe disease results in death within the first year of life. Other types of Pompe disease generally lead to less severe symptoms and longer life span. Pompe disease affects about 1 in 40,000 people in the United States.[8]	Lumizyme (alglucosidase alfa) IV	Avalglucosidase alfa was granted Breakthrough Therapy designation and will provide an alternative therapy option for Pompe disease. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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Lysosomal Storage Disorders (LSDs) - continued	pegunigalsidase alfa IV Chiesi USA/ Protalix BioTherapeutics	The treatment of Fabry disease in adults	Pending FDA approval 04/27/2021	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.[9] Life-threatening complications such as arrhythmias, 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.[10]	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*
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.[11] 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).[12]	**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, pharmacy benefit*

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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.[13] 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.[14] Approximately 8% of DMD patients may be amenable to exon 45 skipping.[15]	**Disease-Modifying Therapy:** Exondys 51 (eteplirsen) IV, Viltepso 53 (viltolarsen) IV, Vyondys 53 (golodirsen) IV – each targets specific 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, medical benefit*

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Neurological Disorders	Aduhelm (aducanumab) IV Biogen/Eisai	The treatment of early Alzheimer's disease (AD), also referred to as mild cognitive impairment due to AD	Pending FDA approval 03/07/2021	AD is a progressive brain disorder that gradually leads to dementia, also referred to as cognitive impairment (loss of memory and thinking and reasoning skills). In early AD, patients experience altered memory and thinking, but are able to perform activities of daily living. As the disease progresses, patients experience memory loss, confusion, and difficulty in completing tasks. Ultimately, patients lose the ability to communicate and care for themselves and generally become bedbound.[16] AD is the most common cause of dementia. An estimated 4.5 million people in the United States have mild cognitive impairment due to AD.[17]	None; current symptomatic therapies are indicated for patients with established AD dementia.	If approved, Aduhelm will provide the first disease-modifying treatment option for patients with early AD. It will be included in Specialty Guideline Management. In November 2020, the Peripheral and Central Nervous System Advisory Committee voted 8 to 1 that a clinical trial did not provide strong evidence of the efficacy of Aduhelm. *Anticipated impact: Incremental spend, 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%. In the United States the prevalence of BC is approximately 3.5 million. An estimated 6% of cases are metastatic at the time of diagnosis.[18]	**IV 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	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.[19,20] MET exon 14 skipping occurs in approximately 3 to 4 % of all newly diagnosed individuals with NSCLC.[21] These individuals tend to be older and have a poor prognosis.	Tabrecta (capmatinib) oral	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, 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.[22] 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.[23] Approximately 15 to 30% of patients relapse and require rescue therapy.[24]	**FDA approved, NCCN supported therapies:** Imbruvica (ibrutinib) oral, Revlimid (lenalidomide) oral + rituximab IV (e.g., Rituxan, Riabni, Ruxience, Truxima) **Select off-label NCCN recommended therapies:** Aliqopa (copanlisib) IV, Copiktra (duvelisib) oral, Gazyva (obinutuzumab) IV + bendamustine IV, rituximab IV, 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, pharmacy benefit*

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Paroxysmal Nocturnal Hemoglobinuria (PNH)	pegcetacoplan SC injection Apellis Pharmaceuticals	The treatment of PNH	Pending FDA approval 05/14/2021	PNH is an acquired disorder in which defective blood cells are produced but are destroyed prematurely. Destruction of red blood cells, or hemolysis, leads to dark-colored urine caused by elimination of hemoglobin into the urine. Chronic hemolysis can lead to other signs and symptoms of PNH including fatigue, rapid heartbeat, chest pain, and shortness of breath. Other complications include blood clots, kidney disease, and infection.[25] PNH is estimated to affect 1 to 5 per million people.[26]	Soliris (eculizumab) IV, Ultomiris (ravulizumab-cwvz) IV	Pegcetacoplan will provide a self-administered treatment option for patients with PNH. It will be included in Specialty Guideline Management. *Anticipated spend: Replacement spend, shift to pharmacy benefit*
Systemic Lupus Erythematosus (SLE)	Luveniq (voclosporin) oral Aurinia Pharmaceuticals	The treatment of lupus nephritis in adults	Pending FDA approval 01/22/2021	SLE is a chronic autoimmune disorder that can affect various organ systems. Lupus nephritis is a complication of SLE that affects the kidneys and may lead to blood in the urine, swelling, high blood pressure, and kidney failure.[27] Approximately 322,000 people in the United States have definite or probable SLE. Although SLE affects people of all ages it is most common in women of childbearing age belonging to minority racial and ethnic groups.[28] Lupus nephritis occurs in up to 80% of children and 50% of adults with SLE.[29]	Benlysta (belimumab) IV, SC Various immunosuppressants may be used off-label (e.g., azathioprine, cyclophosphamide, cyclosporin, mycophenolate mofetil, tacrolimus, corticosteroids)	Luveniq will provide an oral treatment option for lupus nephritis. It will be included in Specialty Guideline Management. *Anticipated spend: Replacement spend, pharmacy benefit*

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