Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q3 2021 – Q4 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 and older	Pending FDA approval 07/27/2021	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 07/15/2021, Rinvoq (upadacitinib) – pending FDA approval 07/19/2021 Numerous topical therapies may be used	Abrocitinib was granted Breakthrough Therapy designation and will provide an oral therapy option for moderate-to-severe AD. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
Growth Hormone Deficiency (GHD)	lonapegsomatropin SC injection Ascendis Pharmaceuticals	The treatment of GHD in pediatrics	Pending FDA approval 09/25/2021	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, and poor development of bones in the middle 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 and somatrogon are additional once weekly, SC, self-administered growth hormone products that will offer a less frequent administration schedule compared to currently available therapies for pediatric GHD. Both agents will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
	somatrogon SC Opko Health/Pfizer	The treatment of GHD in pediatrics	Pending FDA approval 10/19/2021			

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Lysosomal Storage Disorders (LSDs)	avalglucosidase alfa IV Genzyme/Sanofi	The treatment of Pompe disease (glycogen storage disease type II)	Pending FDA approval 08/18/2021	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 U.S.[5]	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*
Neuromuscular	efgartigimod IV Argenyx	The treatment of generalized myasthenia gravis (MG)	Pending FDA approval 12/17/2021	Generalized MG is a chronic autoimmune disorder that causes weakness and fatigue in multiple muscle groups including those of the eyes, face, and jaw, as well as the arms and legs. Approximately 10% of patients may develop myasthenic crisis, a severe and potentially life-threatening complication due to weakness of muscles used in breathing. MG affects approximately 14 to 40 per 100,000 individuals in the U.S.[6]	Acetylcholinesterase inhibitors (neostigmine, pyridostigmine), corticosteroids, Soliris (eculizumab) IV **Off-label agents**: immunosuppressants (e.g., azathioprine, cyclosporine, mycophenolate mofetil, tacrolimus), cyclophosphamide, methotrexate, rituximab, immune globulin IV	Efgartigimod will provide an additional option for patients with inadequate response to conventional MG treatments. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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Oral Oncology	mobocertinib oral Takeda	The treatment of relapsed or refractory advanced non-small cell lung cancer (NSCLC) with EGFR exon 20 insertion mutations	Pending FDA approval 10/26/2021	Lung cancer is the second most common cancer and the leading cause of cancer death among men and women in the U.S. Approximately 582,000 people are living with lung and airway cancer.[7] NSCLC is the most common type of lung cancer, accounting for 84% of all cases.[8] EGFR exon 20 mutations are present in ~0.4% of lung cancer cases in North America.[9]	Rybrevant (amivantamab-vmjw) IV	Mobocertinib was granted Breakthrough Therapy designation and will provide the first oral targeted therapy for NSCLC with EGFR exon 20 mutations. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
Oral Oncology (continued)	pacritinib oral CTI BioPharma	The treatment of myelofibrosis in patients with severe thrombocytopenia	Pending FDA approval 11/30/2021	Myelofibrosis is a disorder of the bone marrow which contains stem cells that will develop into red blood cells, white blood cells, or platelets. Myelofibrosis leads to abnormal blood cell production and scarring of the bone marrow. Symptoms may vary among individuals, but can include weakness, shortness of breath, inability to fight infections, easy bruising, and excessive bleeding.[10] Myelofibrosis occurs in 1.5 in 100,000 people per year in the U.S.[11]	Inrebic (fedratinib), Jakafi (ruxolitinib)	Pacritinib will offer an additional therapy option for patients with myelofibrosis and low platelet levels. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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Psoriasis	bimekizumab SC injection UCB	The treatment of moderate-to-severe plaque psoriasis	Pending FDA approval 11/15/2021	Psoriasis is a chronic autoimmune disease primarily affecting the skin and joints. The most common form, plaque psoriasis, causes raised, thick, scaly patches on the skin that often can itch, cause pain, crack, and bleed.[12] Psoriasis is estimated to affect 8 million Americans, or about 2.4% of the population, with the plaque psoriasis subtype accounting for 80 to 90% of cases.[13] Approximately 20% of patients have moderate-to-severe disease.[14]	**Topical agents:** Various products for mild-to-moderate psoriasis **Oral agent:** Otezla (apremilast) **SC injectable biologic agents:** Cimzia (certolizumab pegol), Cosentyx (secukinumab), Enbrel (etanercept), Humira (adalimumab), Ilumya (tildrakizumab), Siliq (brodalumab), Skyrizi (risankizumab-rzaa), Stelara (ustekinumab), Taltz (ixekizumab), Tremfya (guselkumab) **IV infused biologic agents:** infliximab (Remicade and biosimilar products: Avsola, Inflectra, Renflexis)	If approved, bimekizumab would provide another subcutaneously administered option for the treatment of plaque psoriasis. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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Pulmonary Arterial Hypertension (PAH)	Tyvaso DPI (treprostinil) inhalation MannKind/United Therapeutics	The treatment of PAH and the treatment of pulmonary hypertension associated with interstitial lung disease	Pending FDA approval 10/19/2021	PAH is disorder in which the arteries of the lungs have high blood pressure therefore the heart must work harder to pump blood to the lungs. Common symptoms include severe shortness of breath with exertion, fatigue, chest pain, and fainting. Severe PAH may lead to heart failure.[15] PAH affects 15 to 50 per million people in the U.S. and Europe. PAH most commonly affects women aged 30 to 60.[16]	**Inhaled (nebulized) agents:** Tyvaso (treprostinil), Ventavis (iloprost) **IV agents:** epoprostenol (e.g., Flolan, Veletri), sildenafil (e.g., Revatio), treprostinil (e.g., Remodulin) **Oral agents:** Adempas (riociguat), ambrisentan (e.g., Letairis), bosentan (e.g., Tracleer), Opsumit (macitentan), Orenitram (treprostinil), sildenafil (e.g., Revatio), tadalafil (e.g., Adcirca), Uptravi (selexipag)	If approved, Tyvaso DPI would provide the first inhaled therapy administered via a handheld inhaler device for the treatment of PAH. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
Sleep Disorders	sodium oxybate extended release oral Avadel/Flamel Technologies	The treatment of excessive daytime sleepiness (EDS) and cataplexy in patients with narcolepsy	Pending FDA approval 10/15/2021	Narcolepsy is a chronic sleep disorder in which patients experience chronic and frequent attacks of extreme drowsiness during the day, also referred to as EDS. Other symptoms may include cataplexy (sudden loss of muscle tone triggered by strong emotions), sleep paralysis (temporary inability to move or speak while falling asleep or upon awakening), and hallucinations that can occur with sleep paralysis. Narcolepsy is estimated to affect 1 in 2,000 people; however, the true frequency is unknown as narcolepsy often goes undiagnosed.[17]	**Agents for EDS and/or cataplexy:** various stimulants (e.g., amphetamine-containing products, methylphenidate), armodafinil (e.g., Nuvigil), modafinil (e.g., Provigil), Sunosi (solriamfetol), Xyrem (sodium oxybate), Xywav (oxybate mixed salts), Wakix (pitolisant)	Sodium oxybate extended release will an additional therapy option for patients with narcolepsy. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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Systemic Lupus Erythematosus (SLE)	anifrolumab IV AstraZeneca/ Bristol-Myers Squibb	The treatment of moderate-to-severe SLE in adults	Pending FDA approval 08/05/2021	SLE is a chronic autoimmune disorder in which the immune system attacks its own tissues, causing widespread inflammation and tissue damage that can affect various organ systems (e.g., kidneys, brain, heart, skin, joints).[18] Approximately 322,000 people in the U.S. 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.[19] An estimated 28% of patients have moderate-to-severe disease.[20]	**Oral Agents:** hydroxychloroquine **Injectable Agents:** Benlysta (belimumab) IV, SC Various immunosuppressants are commonly used off-label as part of combination regimens (e.g., azathioprine, mycophenolate mofetil, cyclosporine, methotrexate, glucocorticoids, rituximab, cyclophosphamide, tacrolimus)	Anifrolumab will provide an additional treatment option for SLE. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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