Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q2 2021 – Q3 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 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. 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, 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 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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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.[5] The incidence of NPC is 1 in 100,000 live births. In the U.S. and Europe, there are approximately 1,100 diagnosed patients with NPC.[6]	There are no FDA approved treatments for NPC in the U.S. Zavesca (miglustat) is approved in Europe for NPC and is approved in the U.S. 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 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 U.S.[7]	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.[8] 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.[9]	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*
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 06/07/2021	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.[10] AD is the most common cause of dementia. An estimated 4.5 million people in the U.S. have mild cognitive impairment due to AD.[11]	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*

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Oral Oncology	sotorasib oral Amgen	The treatment of KRAS p.G12C mutant advanced non-small cell lung cancer (NSCLC) following at least one prior systemic therapy in adults	Pending FDA approval 08/16/2021	Lung cancer is the second most common cancer and the leading cause of cancer death among men and women in the U.S. Approximately 558,000 people are living with lung cancer.[12] NSCLC is the most common type of lung cancer, accounting for 84% of all cases.[13] KRAS p.G12C mutations occur in approximately 13% of NSCLC patients. Presence of these mutations is prognostic for poor survival and is often associated with resistance to targeted therapies.[14]	Various IV agents used off-label, including: Cyramza (ramucirumab) + docetaxel, Keytruda (pembrolizumab), Opdivo (nivolumab), Tecentriq (atezolizumab), conventional chemotherapy	Sotorasib was granted Breakthrough Therapy designation and will be the first FDA approved agent targeting KRAS mutations for NSCLC. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, shift to pharmacy benefit*
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.[15] PNH is estimated to affect 1 to 5 per million people.[16]	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 impact: Replacement spend, shift to pharmacy benefit*

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Psoriasis	bimekizumab SC injection UCB	The treatment of moderate-to-severe plaque psoriasis	Pending approval 07/15/2021	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.[17] 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.[18] Approximately 20% of patients have moderate-to-severe disease.[19]	**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 treatment of plaque psoriasis. 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 approval 07/05/2021	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).[20] 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.[21] An estimated 28% of patients have moderate-to-severe disease.[22]	**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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