Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q1 2022 – Q2 2022

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
Amyloidosis	vutrisiran SC Alnylam	The treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR) in adults	Pending FDA approval 04/14/2022	hATTR is a rare, progressive and fatal disease caused by mutations in the transthyretin gene. Mutations that occur are predominantly associated with either polyneuropathy or cardiomyopathy. Signs and symptoms of hATTR may include tingling, numbness, or pain in the hands or feet, difficulty walking, loss of balance, irregular heartbeat, and dizziness from low blood pressure. The U.S. prevalence of hATTR amyloidosis with polyneuropathy is estimated to be 10,000 to 15,000 patients, although fewer than 3,000 have been diagnosed.[2]	Onpattro (patisiran) IV, Tegsedi (inotersen) SC	Vutrisiran would provide an additional therapy option with a less frequent, quarterly administration schedule. It will be administered by a health care provider. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, partial shift from pharmacy benefit*
Atopic Dermatitis (AD)	Cibinqo (abrocitinib) oral Pfizer	The treatment of moderate-to-severe AD in patients aged 12 and older	Pending FDA approval 1Q 2022	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.[3] Approximately 40% of patients have moderate-to-severe disease.[4]	Adbry (tralokinumab-ldrm) SC, Dupixent (dupilumab) SC **Approved oral agents seeking label expansions for AD**: Olumiant (baricitinib) – pending FDA approval 1Q 2022, Rinvoq (upadacitinib) – pending FDA approval 1Q 2022 Numerous topical therapies may be used.	Cibinqo was granted Breakthrough Therapy designation and would provide an oral therapy option for moderate-to-severe AD. Due to drug class safety concerns, use of Cibinqo will likely be limited to second-line use. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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Cardiac Disorders	mavacamten oral Bristol Myers Squibb	The treatment of hypertrophic obstructive cardiomyopathy (HOCM)	Pending FDA approval 04/28/2022	HOCM is a type of genetic heart disease that causes the heart to contract with greater force. This results in an abnormally thickened heart muscle, ultimately leading to the heart's inability to relax normally and fill with blood to effectively pump to the rest of the body. Patients with HOCM are typically symptomatic and may experience chest pain, shortness of breath, fatigue, irregular heartbeat, dizziness and lightheadedness. 1 in 500 people are affected by hypertrophic cardiomyopathy (HCM), but only approximately 15% are formally diagnosed.[5,6] Approximately 70% of patients with HCM have left ventricular tract outflow obstruction, or HOCM.[7]	No FDA approved agents. **Off-label oral agents**: beta-blockers, calcium channel blockers, Norpace (e.g., disopyramide)	Mavacamten was granted Breakthrough Therapy designation and would be the first disease-modifying drug therapy approved for the treatment of HOCM and represents an add-on therapy to standard of care in patients with symptomatic disease. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, pharmacy benefit*
Growth Hormone Deficiency (GHD)	somatrogon SC Opko Health/ Pfizer	The treatment of GHD in pediatrics	Pending FDA approval 01/19/2022	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.[8]	**SC, daily administered somatropin (recombinant human growth hormone) agents:** Genotropin, Humatrope, Norditropin, Nutropin AQ, Omnitrope, Saizen, Zomacton **Long-acting growth hormone agents:** Skytrofa (lonapegsomatropin-tcgd), Sogroya (somapacitan-beco) – indicated for adult GHD	Somatrogon is an additional once weekly, SC, self-administered growth hormone product that would provide a less frequent administration schedule compared to daily 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)	lenacapavir oral and SC Gilead	The treatment of HIV infection in heavily treatment-experienced patients, in combination with other antiretroviral therapy (ART)	Pending FDA approval 02/28/2022	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. If not treated, HIV can lead to acquired immunodeficiency syndrome. An estimated 1.2 million people are living with HIV in the U.S. and approximately 13% are unaware they have the disease.[9] The prevalence of treatment-experienced patients with limited treatment options is 0.8%.[10]	**ART for treatment-experienced patients with resistance**: Aptivus (tipranavir) oral, Fuzeon (enfuvirtide) SC, Intelence (etravirine) oral, Rukobia (fostemsavir) oral, Trogarzo (ibalizumab-uiyk) IV	Lenacapavir was granted Breakthrough Therapy designation and would provide an alternative therapy option for heavily treatment-experienced HIV patients. *Anticipated impact: Replacement spend, medical benefit*

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Oral Oncology	pacritinib oral CTI BioPharma	The treatment of myelofibrosis in patients with severe thrombocytopenia	Pending FDA approval 02/28/2022	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.[11] Myelofibrosis occurs in 1.5 in 100,000 people per year in the U.S.[12]	Inrebic (fedratinib), Jakafi (ruxolitinib)	Pacritinib would provide 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*
	parsaclisib oral Incyte	The treatment of relapsed or refractory (R/R) marginal zone lymphoma (MZL) in adults who have received at least one prior anti-CD20-based regimen	Pending FDA approval 04/30/2022	MZL and MCL are types of non-Hodgkin's B-cell lymphomas. These are blood cancers that begin in the lymphatic system. Approximately 60,000 people in the U.S. are living with MZL. About 20 to 30% of these patients will develop resistance to treatment.[13,14,15] Approximately 44,000 people in the U.S. are living with MCL. About 8 to 17% of patients receive second-line therapy.[16,17,18]	**Oral agents**: Brukinsa (zanubrutinib), Copiktra (duvelisib), Imbruvica (ibrutinib), Revlimid (lenalidomide) + rituximab IV, Ukoniq (umbralisib), Zydelig (idelalisib) **IV agents**: Aliqopa (copanlisib), various chemotherapy regimens + rituximab or obinutuzumab, Zevalin (ibritumomab tiuxetan)	Parsaclisib would provide an additional second-line or later therapy for each indication. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
		The treatment of mantle cell lymphoma (MCL) in adults who have received at least one prior therapy			**Oral agents**: Brukinsa, Calquence (acalabrutinib), Imbruvica +/- rituximab IV, Revlimid + rituximab IV **IV agents**: various chemotherapy regimens + rituximab, Tecartus (brexucabtagene autoleucel)	

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Paroxysmal Nocturnal Hemoglobinuria (PNH)	Ultomiris (ravulizumab-cwvz) SC Alexion	The treatment of atypical hemolytic uremic syndrome (aHUS) in treatment-naive adults and the treatment of PNH in patients aged 12 and older	Pending FDA approval 05/15/2022	aHUS is a rare disease characterized by low levels of circulating red blood cells, low platelet count and kidney dysfunction. aHUS may become chronic, in which case patients may develop serious complications such as severe high blood pressure and kidney failure. aHUS is estimated to affect 2 in 1 million people.[19] PNH is a rare, acquired, life-threatening blood disease in which red blood cells break apart prematurely. The destruction of red blood cells leads to the presence of hemoglobin in the urine. Patients are susceptible to developing repeated, potentially life-threatening blood clots and may also have some degree of underlying bone marrow dysfunction. PNH is estimated to affect 12 to 13 in 1 million people.[20]	Empaveli (pegcetacoplan) SC (PNH only), Soliris (eculizumab) IV, Ultomiris IV	Ultomiris would provide an additional therapy option that is self-administered once weekly via an on-body device. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, partial shift from medical benefit*

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Psoriasis	bimekizumab SC UCB	The treatment of moderate-to-severe plaque psoriasis in adults	Pending FDA approval 01/15/2022	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.[21] 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.[22] Approximately 20% of patients have moderate-to-severe disease.[23]	**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)	Bimekizumab would provide another SC 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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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 01/15/2022	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.[24]	**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 would provide 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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