Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q2 2022 – Q3 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 subcutaneous (SC) Alnylam	The treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR) in adults	Pending FDA approval 07/14/2022	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) intravenous (IV), Tegsedi (inotersen) SC	Vutrisiran would provide an additional therapy option with a less frequent, quarterly administration schedule. It will be administered by a healthcare provider. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, partial shift from pharmacy benefit*
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 is affected by hypertrophic cardiomyopathy (HCM) but only approximately 15% are formally diagnosed.[3,4] Approximately 70% of patients with HCM have left ventricular tract outflow obstruction, or HOCM.[5]	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. Mavacamten would be an add-on therapy to the standard of care in patients with symptomatic disease. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, pharmacy benefit*

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Diabetes	teplizumab IV Provention Bio	The delay of type 1 diabetes (T1D) in high-risk individuals aged 8 to 45 years	Pending FDA approval 08/17/2022	T1D is a chronic disorder in which the pancreas does not make insulin or produces an insufficient amount of insulin. Insulin is a hormone that helps blood glucose (sugar) enter the cells of the body where it can be used for energy. Without insulin, blood sugar can't get into cells and builds up in the bloodstream. High blood sugar is damaging to the body and causes many of the symptoms and complications of diabetes.[6] T1D affects approximately 1.6 million in the U.S.[7] Additionally, an estimated 300,000 have the early stages of the disease and show no symptoms.[8] These patients are said to have presymptomatic T1D, and approximately 100,000 to 150,000 of them are considered to be at high risk for progressing to symptomatic (insulin-dependent) T1D.[9]	None; current insulin-based therapies are focused on the treatment of T1D, not prevention	Teplizumab was granted Breakthrough Therapy designation and would be the first approved therapy to delay T1D. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, medical benefit (preventive therapy may result in T1D medical cost avoidance)*

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Lysosomal Storage Disorders (LSDs)	cipaglucosidase alfa IV Amicus Therapeutics	The combination treatment of late-onset Pompe disease (glycogen storage disease type II) in adults	Pending FDA approval 07/29/2022	Pompe disease is a rare, inherited LSD leading to the accumulation of glycogen, a complex sugar, in muscles as well as other organs and tissues. There are three different types of Pompe disease: classic infantile and non-classic infantile-onset (IOPD), and late-onset (LOPD). Each type differs in severity and the age at which symptoms appear. In IOPD, symptoms generally begin a few months after birth and the disease is more severe. In LOPD, symptoms generally begin later in childhood, adolescence, or even adulthood, and are less severe.[10] Progressing more slowly than infantile types, LOPD primarily affects skeletal muscles leading to weakness, especially in the legs and the trunk. As the disorder advances, the muscles that control breathing are affected, which can lead to respiratory failure if left untreated. LOPD affects about 1 in 57,000 people in the U.S.[11]	Lumizyme (alglucosidase alfa) IV, Nexviazyme (avalglucosidase alfa-ngpt) IV	The combination of cipaglucosidase and miglustat was granted Breakthrough Therapy designation and will provide an alternative therapy option. It will be included in Specialty Guideline Management. *Anticipated impact:* *cipaglucosidase: Replacement spend, medical benefit* *miglustat: Incremental spend pharmacy benefit*
	miglustat oral Amicus Therapeutics		Pending FDA approval 05/29/2022			

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Multiple Sclerosis (MS)	ublituximab IV TG Therapeutics	The treatment of relapsing-remitting MS	Pending FDA approval 09/28/2022	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 U.S. 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), Ponvory (ponesimod), Tecfidera (dimethyl fumarate), Vumerity (diroximel fumarate), Zeposia (ozanimod)	Ublituximab is in the same drug class as Ocrevus and Kesimpta, which would provide an additional therapy option. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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Neuromuscular Disorders	edaravone oral Mitsubishi Tanabe Pharma	The treatment of amyotrophic lateral sclerosis (ALS; also known as Lou Gehrig's disease)	Pending FDA approval 05/12/2022	ALS is a fatal disorder characterized by progressive destruction of motor neurons, the nerves that control voluntary muscles. Patients eventually lose the ability to eat, speak, walk, and breathe on their own. [14] Approximately 16,500 Americans have ALS.[15]	riluzole oral products (i.e., Rilutek, Exservan, Tiglutik and generics), Radicava (edaravone) IV	Edaravone has the same active ingredient as Radicava IV and would provide an oral alternative therapy option. It will be included in Specialty Guideline Management *Anticipated impact: Replacement spend, shift from medical benefit*
	sodium phenylbutyrate/ taurursodiol oral Amylyx		Pending FDA approval 06/29/2022			Sodium phenylbutyrate/ taurursodiol may be an alternative or an add-on to existing therapies and would provide an additional therapy option. In March 2022, the Peripheral and Central Nervous System Drugs Advisory Committee voted 4 to 6 that the single clinical trial did not provide strong evidence of efficacy. It will be included in Specialty Guideline Management *Anticipated impact: Incremental spend, pharmacy benefit*

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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.[16] 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.[17]	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 04/15/2022	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.[18] 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.[19] Approximately 20% of patients have moderate-to-severe disease.[20]	**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 has a broader target than Cosentyx and Taltz, and would provide another SC option. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
	deucravacitinib oral Bristol Myers Squibb		Pending FDA approval 09/10/2022			Deucravacitinib would provide an additional oral option. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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Pulmonary Arterial Hypertension (PAH)	Tyvaso dry powder inhaler (DPI) (treprostinil) inhalation MannKind/United Therapeutics	The treatment of PAH and the treatment of pulmonary hypertension associated with interstitial lung disease	Pending FDA approval 05/27/2022	PAH is a disorder in which the arteries of the lungs have high blood pressure therefore the heart must work harder to pump blood to the lungs. Interstitial lung disease (ILD) causes scarring (fibrosis) of the lungs, which can lead to PAH. Severe PAH may lead to heart failure.[21] ILD is an umbrella name for a multitude of diseases which include lung damage arising from autoimmune disorders, genetic mutations, radiation, and hazardous materials exposure, among others.[22] Common symptoms include severe shortness of breath with exertion, fatigue, chest pain, and fainting. PAH affects 15 to 50 per million people in the U.S. and Europe. PAH most commonly affects women aged 30 to 60.[23] The presence of pulmonary hypertension in patients with ILD varies widely, but studies found an overall prevalence of 25% in those referred for a lung transplant.[24]	**Inhaled (nebulized) agents:** Tyvaso (treprostinil), Ventavis (iloprost) **IV agents:** epoprostenol (e.g., Flolan, Veletri), sildenafil (e.g., Revatio), treprostinil (e.g., Remodulin), Uptravi (selexipag) **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)	Tyvaso DPI would provide a portable, handheld inhaler device option. 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 04/15/2022	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.[25]	**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. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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