Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q3 2022 – Q4 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
Endocrine Disorders - Other	teplizumab IV Provention Bio	The delay of type 1 diabetes (T1D) in high-risk individuals aged 8 to 45 years	Pending FDA approval 11/17/2022	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.[2] T1D affects approximately 1.6 million in the U.S.[3] Additionally, an estimated 300,000 individuals have the early stages of disease and show no symptoms.[4] 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.[5]	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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Hepatitis D	Hepcludex (bulevirtide) SC Gilead	The treatment of chronic hepatitis delta virus (HDV) in adults with compensated liver disease	Pending FDA approval 07/19/2022	HDV is a liver infection that occurs only in individuals infected with hepatitis B virus (HBV). Infection with both HBV and HDV can be acquired at the same time (coinfection) or after HBV infection has occurred (superinfection). Chronic HDV infection causes more severe liver disease and leads to a faster progression of permanent liver scarring, an increased risk of liver cancer, and death compared to HBV infection alone.[6] It is estimated that more than 230,000 individuals in the U.S. and Europe are living with HDV; however less than 20% of cases have been diagnosed.[7]	None **Off-label:** peginterferon alfa SC	Hepcludex was granted Breakthrough Therapy designation and would be the first approved therapy for HDV. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, pharmacy benefit*
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 12/27/2022	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 left untreated, 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.[8] The prevalence of treatment-experienced patients with limited treatment options is 0.8%.[9]	**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 with an extended dosing interval for heavily treatment-experienced HIV patients. *Anticipated impact: Replacement spend, medical benefit*

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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 10/29/2022	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 would provide an alternative therapy option. It will be included in Specialty Guideline Management. *Anticipated impact:* *cipaglucosidase alfa: Replacement spend, medical benefit* *miglustat: Incremental spend, pharmacy benefit*
	miglustat oral Amicus Therapeutics		Pending FDA approval 08/29/2022			

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Multiple Sclerosis (MS)	ublituximab IV TG Therapeutics	The treatment of relapsing-remitting MS	Pending FDA approval 12/28/2022	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 years 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 IM, Rebif SC (interferon beta-1a), Betaseron/Extavia (interferon beta-1b) SC, glatiramer (e.g., Copaxone) SC, Kesimpta (ofatumumab) SC, Lemtrada (alemtuzumab) IV, Ocrevus (ocrelizumab) IV, Plegridy (peginterferon beta 1a) IM/SC, Tysabri (natalizumab) IV **Oral Agents:** Aubagio (teriflunomide), Bafiertam (monomethyl fumarate), dimethyl fumarate (e.g., Tecfidera), Gilenya (fingolimod), Mavenclad (cladribine), Mayzent (siponimod), Ponvory (ponesimod), Vumerity (diroximel fumarate), Zeposia (ozanimod)	Ublituximab is in the same drug class as Ocrevus and Kesimpta, and would provide an additional therapy option. Ublituximab will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, medical benefit*

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Movement Disorders	sodium phenylbutyrate/ taurursodiol oral Amylyx	The treatment of amyotrophic lateral sclerosis (ALS; also known as Lou Gehrig's disease)	Pending FDA approval 09/29/2022	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, Radicava ORS (edaravone) oral	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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Oral Oncology	adagrasib oral Mirati Therapeutics	The treatment of KRAS G12C mutated advanced non-small cell lung cancer (NSCLC) following at least 1 prior systemic therapy in adults	Pending FDA approval 12/14/2022	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. NSCLC is the most common type of lung cancer, accounting for about 85% of all cases.[16] KRAS G12C mutations occur in approximately 13% of NSCLC patients. Presence of these mutations is prognostic of poor survival and is often associated with resistance to targeted therapies.[17]	Lumakras (sotorasib) oral	Adagrasib was granted Breakthrough Therapy designation and would provide an additional oral therapy option. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement 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 07/15/2022	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.[18] 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.[19]	Empaveli (pegcetacoplan) SC (PNH only), Soliris (eculizumab) IV, Ultomiris IV	Ultomiris SC 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	deucravacitinib oral Bristol Myers Squibb	The treatment of moderate-to-severe plaque psoriasis in adults	Pending FDA approval 09/10/2022	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.[20] 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.[21] Approximately 20% of patients have moderate-to-severe disease.[22]	**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)	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)	Yutrepia (treprostinil) Liquidia	The treatment of PAH	Pending FDA approval 10/27/2022	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.[23] PAH affects 15 to 50 per million people in the U.S. and Europe. PAH most commonly affects women aged 30 to 60 years.[24]	**Inhaled (dry powder inhalation) agent**: Tyvaso DPI (treprostinil) **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)	Yutrepia would provide an additional portable, handheld inhaler device option. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*

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