Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q4 2022–Q1 2023

CVS Health

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/ Sanofi	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. Prolonged high blood sugar is damaging to the body and causes many of the symptoms and complications of diabetes.[2] T1D affects approximately 1.9 million people 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)*
Hemophilia	efanesoctocog alfa IV Sanofi	The prevention of bleeding episodes in patients aged 12 years and older with severe hemophilia A	Pending FDA approval 02/28/2023	Hemophilia A is a genetic disorder caused by missing or defective factor VIII (FVIII), a clotting protein. People with hemophilia bleed longer than other people. The frequency and severity of bleeding episodes depends on the quantity of FVIII the person naturally produces. Hemophilia occurs in approximately 1 in 5,600 live male births. There are between 30,000 and 33,000 males with hemophilia in the U.S. Hemophilia A makes up 80% to 85% of total cases. More than half of patients diagnosed with hemophilia A have the severe form.[6]	Traditional FVIII replacement therapies, Hemlibra (emicizumab-kxwh)	Efanesoctocog alfa was granted Breakthrough Therapy designation and would be an additional factor replacement option with a less frequent administration schedule compared to current FVIII agents. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, primarily pharmacy benefit*

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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 11/19/2022	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.[7] 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.[8]	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.[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 with an extended dosing interval for heavily treatment-experienced HIV patients. *Anticipated impact: Replacement spend, medical benefit*

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Inflammatory Bowel Disease, Psoriasis, Rheumatoid Arthritis	Amjevita (adalimumab-atto) SC Amgen	The treatment of rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis, ulcerative colitis, and plaque psoriasis in adults, the treatment of juvenile idiopathic arthritis in patients aged 2 years and older, and the treatment of Crohn's disease in patients aged 6 years and older (biosimilar of Humira)	Approved 09/23/2016	A biosimilar is a biologic product that has been demonstrated to be highly similar to an already FDA-approved biologic product (known as the reference product). A biosimilar does not have clinically meaningful differences in safety and effectiveness as compared to the reference product. Only minor differences in clinically inactive components are allowed.[11]	Humira (adalimumab) SC Numerous other products (both approved and off-label) can be used in the management of the various autoimmune disease states that Humira is used to treat.	Amjevita will be the first biosimilar of Humira to launch in the U.S. Six additional adalimumab biosimilar products have been approved and at least three others are awaiting approval. All of these biosimilars may be available in 2023. Amjevita and other adalimumab biosimilars will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (potential for decreased spend), pharmacy benefit*
Lysosomal Storage Disorders (LSDs)	cipaglucosidase alfa IV Amicus Therapeutics miglustat oral Amicus Therapeutics	The combination treatment of late-onset Pompe disease (glycogen storage disease type II) in adults	Pending FDA approval 10/29/2022 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.[12] 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.[13]	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*

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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.[14] 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).[15]	**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*
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 third 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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Oral Oncology	elacestrant oral Radius Health	The treatment of advanced or metastatic estrogen receptor-positive, HER2-negative breast cancer	Pending FDA approval 02/17/2023	Breast cancer is the second most common cancer and the second leading cause of cancer-related death in women in the U.S. The lifetime risk of developing breast cancer is approximately 13% for U.S. women.[18] Hormones such as estrogen and progesterone can promote the growth of hormone receptor (HR)-positive breast cancers. HER2 is a protein that promotes the growth of cancer. HER2-positive breast cancers tend to be more aggressive. The HR-positive, HER2-negative subtype accounts for approximately 68% of breast cancer cases.[19]	Antihormonal therapies (e.g., fulvestrant IM, anastrozole oral, exemestane oral, letrozole oral, tamoxifen oral) with or without Afinitor (everolimus) oral	Elacestrant may be the first approved agent in a new class called the selective estrogen receptor degraders and will provide an additional oral therapy option for breast cancer. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
Oral Oncology	pirtobrutinib oral Eli Lilly	The treatment of relapsed or refractory mantle cell lymphoma (MCL) in patients previously treated with a Bruton's tyrosine kinase (BTK) inhibitor	Pending FDA approval 02/04/2023	Non-Hodgkin's lymphoma (NHL) refers to a group of blood cancers that develop in lymphocytes, a type of white blood cell. MCL accounts for 3% to 8% of cases with NHL. Complications of MCL can include low blood cell counts, as well as gastrointestinal, pulmonary, or central nervous system involvement.[20]	**Oral BTK Inhibitors:** Brukinsa (zanubrutinib), Calquence (acalabrutinib), Imbruvica (ibrutinib) **Other Agents:** bortezomib IV/SC (e.g., Velcade), lenalidomide oral (e.g., Revlimid), Tecartus (brexucabtagene autoleucel) IV	Pirtobrutinib will provide an additional oral therapy option for previously treated patients with MCL. It will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend, pharmacy benefit*
Renal Disease	sparsentan oral Travere Therapeutics	The treatment of immunoglobulin A nephropathy in adults	Pending FDA approval 02/17/2023	Immunoglobulin A nephropathy (IgAN) is a chronic, slowly progressive kidney disease that occurs when an antibody, immunoglobulin A, accumulates in the kidneys and in turn leads to inflammation and kidney damage. Approximately 30% of IgAN patients will develop end-stage renal disease.[21] There are approximately 60,000 Americans with IgAN. About 45% of IgAN patients do not respond to initial angiotensin-converting enzyme inhibitor (ACEI)/ angiotensin receptor blocker (ARB) treatment.[22,23]	**Immunosuppressive Agents:** Tarpeyo (budesonide) **Off-label Immunosuppressive Agents:** Oral Agents: systemic glucocorticoids, mycophenolate mofetil, cyclosporine, tacrolimus, azathioprine, cyclophosphamide, leflunomide, hydroxychloroquine IV Agents: rituximab, cyclophosphamide, other cytotoxic agents **Off-label, Oral Non-immunosuppressive Agents:** ACEIs, ARBs, mineralocorticoid receptor antagonists, SGLT2 inhibitors	Sparsentan would offer an additional, later-line option for those with inadequate response to first-line treatment. It will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend, pharmacy benefit*

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