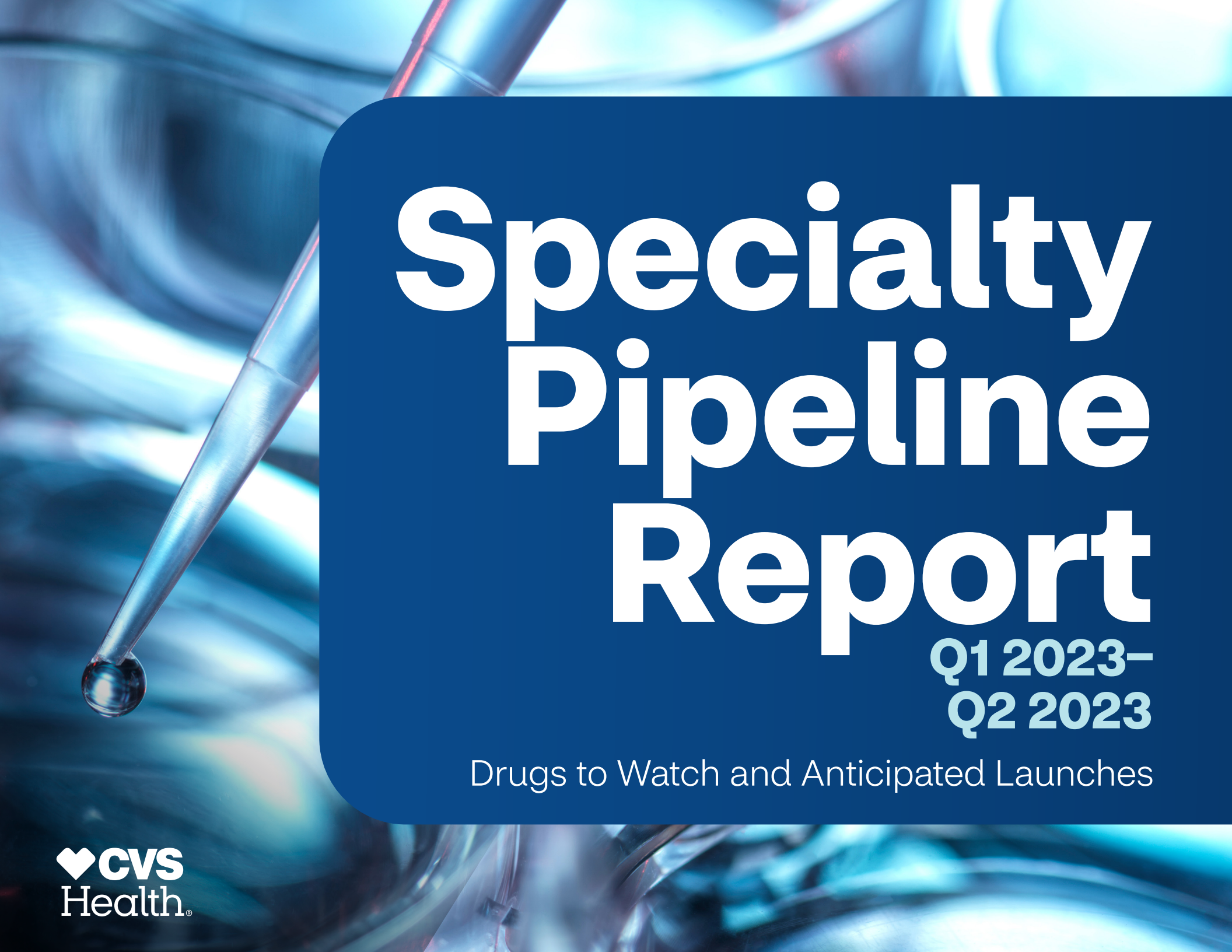

Specialty Pipeline Report

Q1 2023–Q2 2023

Drugs to Watch and Anticipated Launches

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 FDA-APPROVED THERAPIES*	COMMENTS
Alopecia Areata (AA)	**ritlecitinib** oral Pfizer	The treatment of AA in patients aged 12 years and older	Pending FDA approval 06/09/2023	AA is an autoimmune disorder that develops when the immune system attacks the body's hair follicles, causing hair to fall out. This hair loss often occurs in patches on the scalp, but it can also affect other areas of the body including the legs and face. AA affects approximately 6.8 million people in the U.S.[2] The prevalence of moderate-to-severe disease is ~0.09%.[3]	Olumiant (baricitinb) oral (approved for adults)	Ritlecitinib was granted Breakthrough Therapy designation and would provide an additional therapy option for AA in adults and would provide the first therapy option for patients aged 12 to 17 years. It will be included in Specialty Guideline Management. **Anticipated impact:** Incremental spend, pharmacy benefit
Anemia	**daprodustat** oral GSK	The treatment of anemia associated with dialysis-dependent (DD) and non-dialysis-dependent (NDD) chronic kidney disease (CKD)	Pending FDA approval 02/01/2023	Anemia is common in people with kidney disease. Anemia happens when there are not enough healthy red blood cells in the body. People with CKD may start to have anemia in the early stages of CKD, but it is most common in later stages (moderate-to-severe disease). Signs or symptoms of anemia may include dizziness, loss of concentration, chest pain, shortness of breath and fatigue. About 37 million U.S. adults have CKD with ~7% having moderate-to-severe disease.[4] Approximately 11% of later stage NDD-CKD patients and ~77% of end stage renal disease patients receive erythropoietin stimulating agents (ESAs).[5,6,7]	Epoetin alfa (e.g., Epogen, Procrit, Retacrit [biosimilar]) IV/SC; Aranesp (darbepoetin alfa) IV/SC; Mircera (methoxy polyethylene glycol-epoetin beta) IV/SC	Daprodustat would provide an orally administered alternative to injectable ESAs. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, shift from medical benefit

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Hemophilia	**Altuviiio (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 whereas hemophilia B is caused by missing or defective factor IX (FIX). People with hemophilia bleed longer than other people. The frequency and severity of bleeding episodes depends on the quantity of FVIII or FIX 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.[8]	**IV:** Traditional FVIII replacement therapies **SC:** 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, pharmacy benefit
	concizumab SC Novo Nordisk	The prevention of bleeding episodes in patients with hemophilia A and B with inhibitors	Pending FDA approval 04/23/2023		**IV:** Traditional FVIII and FIX replacement therapies, FEIBA (anti-inhibitor coagulant complex), NovoSeven (coagulation factor VIIa) **SC:** Hemlibra (hemophilia A only)	Concizumab was granted Breakthrough Therapy designation for hemophilia B with inhibitors and would provide an additional therapy option for hemophilia A and the first subcutaneously administered therapy option for hemophilia B. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit

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Inflammatory Bowel Disease	**mirikizumab** IV and SC Eli Lilly	The treatment of moderate-to-severe ulcerative colitis (UC)	Pending FDA approval 03/10/2023	UC is a chronic inflammatory disease of the large intestine, also called the colon, that affects the lining of the colon and causes small sores, or ulcers, to form. The ulcers produce pus and mucous, which cause abdominal pain and the need to frequently empty the colon. Symptoms can range from mild to severe. It is estimated that 600,000 to 900,000 Americans have UC. About 20% have moderate disease and ~1–2% have severe disease.[9,10]	**IV:** Entyvio (vedolizumab), infliximab (Remicade and biosimilar products Avsola, Inflectra, Renflexis), Stelara (ustekinumab) **Oral:** Rinvoq (upadacitinib), Xeljanz/Xeljanz XR (tofacitinib), Zeposia (ozanimod) **SC:** Humira (adalimumab), Simponi (golimumab)	Mirikizumab would provide an additional once monthly subcutaneously administered treatment option. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit
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 to Humira)	Approved 09/23/2016 Anticipated launch 01/31/2023	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 to Humira to launch in the U.S. Seven additional adalimumab biosimilar products have been approved and at least two others are awaiting FDA 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

Amjevita will be the first biosimilar to Humira to launch in the U.S.

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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 01/29/2023 Pending FDA approval 01/29/2023	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
Lysosomal Storage Disorders (LSDs)	**pegunigalsidase** IV Chiesi USA/ Protalix BioTherapeutics	The treatment of Fabry disease in adults	Pending FDA approval 05/09/2023	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.[14] 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.[15]	Fabrazyme (agalsidase beta) IV, Galafold (migalastat) oral - limited to those with an amenable genetic variation	Pegunigalsidase alfa would provide an alternative treatment option with the potential for a less frequent infusion schedule than Fabrazyme. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, medical benefit

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Muscular Dystrophy	**vamorolone** oral Santhera Pharmaceuticals/ ReveraGen BioPharma	The treatment of Duchenne muscular dystrophy (DMD) in ambulant boys aged 4 years and older	Pending FDA approval 06/27/2023	DMD is a rare, genetic disorder that is characterized by progressive muscle damage and weakness. DMD occurs primarily in males, though in rare cases may affect females. Over time, children with DMD develop complications such as loss of walking ability and other motor skills. Serious life-threatening complications may ultimately develop including disease of the heart muscle and breathing difficulties. DMD is an irreversible disease; however, some management options can help to slow disease progression. DMD occurs in approximately 1 in 3,500 live male births.[16]	Emflaza (deflazacort) oral, prednisone oral	Vamorolone would provide an additional corticosteroid option with a potentially safer adverse event profile compared to prednisone. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit
Neuromuscular	**efgartigimod** SC Argenx	The treatment of generalized myasthenia gravis (MG) in adults	Pending FDA approval 03/20/2023	Generalized MG is a chronic autoimmune disorder that causes weakness and fatigue in multiple muscle groups including those of the eyes, face, and jaw, as well as the arms and legs. MG affects approximately 14 to 40 per 100,000 individuals in the U.S.[17] The prevalence of refractory MG ranges from ~10% to 20%.[18]	Soliris (eculizumab) IV, Ultomiris (ravulizumab-cwvz) IV, Vyvgart (efgartigimod) IV	Efgartigimod would provide the first subcutaneously administered treatment option for patients with inadequate response to conventional MG treatments. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, shift to pharmacy benefit
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.[19] 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.[20]	Antihormonal therapies (e.g., fulvestrant IM, anastrozole oral, exemestane oral, letrozole oral, tamoxifen oral) with or without Afinitor (everolimus) oral	Elacestrant would 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

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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.[21]	**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 would 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
Psoriasis	**bimekizumab** SC UCB	The treatment of moderate-to-severe plaque psoriasis	Pending FDA approval 05/22/2023	Psoriasis is a chronic autoimmune disorder 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.[22] Psoriasis is estimated to affect 8 million Americans, or about 2.4% of the population, with the plaque psoriasis subtype accounting for 80-90% of cases.[23] Approximately 20% of patients have moderate-to-severe disease.[24]	**IV:** infliximab (Remicade and biosimilars) **Oral:** Otezla (apremilast), Sotyktu (deucravacitinib) **SC:** Cimzia (certolizumab pegol), Cosentyx (secukinumab), Enbrel (etanercept), Humira (adalimumab), Ilumya (tildrakizumab-asmn), Siliq (brodalumab), Skyrizi (risankizumab-rzaa), Stelara (ustekinumab), Taltz (ixekizumab), Tremfya (guselkumab)	Bimekizumab would provide an additional subcutaneously administered therapy option for plaque psoriasis. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit

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Renal Disease	**sparsentan** oral Travere Therapeutics	The treatment of immunoglobulin A nephropathy (IgAN) in adults	Pending FDA approval 02/17/2023	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.[25] 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.[26,27]	**Immunosuppressive Agents:** Tarpeyo (budesonide) oral **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

1. RxPipeline, January 2023.
2. National Alopecia Areata Foundation. Available at https://www.naaf.org/faqs. Accessed 12/29/2022.
3. Benigno M, Anastassopoulos KP, Mostaghimi A, et al. A large cross-sectional survey study of the prevalence of alopecia areata in the United States. Clin Cosmet Investig Dermatol 2020;13:259-66.
4. United States Renal Data System: CKD in the General Population. Available at https://adr.usrds.org/2021/chronic-kidney-disease/1-ckd-in-the-general-population. Accessed 04/22/2022.
5. Anemia Prevalence and Treatment Rates in Stage 3-5 Non-Dialysis-Dependent Chronic Kidney Disease Patients. Available at http://www.cdrg.org/media/1390/2016-amcp_gilbertson_anemia-prev-tx-rates.pdf. Accessed 04/19/2019.
6. United States Renal Data System: ESRD in the US. Available at https://adr.usrds.org/2021/end-stage-renal-disease/1-incidence-prevalence-patient-characteristics-and-treatment-modalities. Accessed 04/22/2022.
7. United States Renal Data System: ESRD in the US. Available at https://adr.usrds.org/2021/end-stage-renal-disease/3-clinical-indicators-and-preventive-care. Accessed 04/22/2022.
8. National Hemophilia Foundation – Hemophilia A. Available at https://www.hemophilia.org/bleeding-disorders-a-z/types/hemophilia-a. Accessed 09/20/2022.
9. National Institute of Diabetes and Digestive and Kidney Diseases. Available at https://www.niddk.nih.gov/health-information/digestive-diseases/ulcerative-colitis/definition-facts. Accessed 12/20/2022.
10. Crohn's and Colitis Foundation of America. Available at https://www.crohnscolitisfoundation.org/sites/default/files/2019-02/Updated%20IBD%20Factbook.pdf. Accessed 12/20/2022.
11. US Food and Drug Administration. Biosimilar and interchangeable products. Available at https://www.fda.gov/drugs/biosimilars/overview-health-care-professionals. Accessed 01/10/2023.
12. MedlinePlus. Pompe disease. Available at https://medlineplus.gov/genetics/condition/pompe-disease/#synonyms. Accessed 03/24/2022.
13. American Association of Neuromuscular & Electrodiagnostic Medicine and MedLogix Communications, LLC. Late-onset Pompe disease. Presentation, diagnosis, and management: a CME monograph. Available at https://www.aanem.org/mxonline/resources/downloads/products/ED01.pdf. Accessed 03/31/2022.
14. Fabry Disease News. Available at https://fabrydiseasenews.com/what-is-fabry-disease/. Accessed 10/06/2020.
15. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/fabry-disease/. Accessed 10/06/2020.
16. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/#:~:text=The%20birth%20prevalence%20is%20estimated,individuals%20in%20the%20United%20States. Accessed 12/20/2022.
17. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/myasthenia-gravis/. Accessed 06/29/2021.
18. Therapeutic Advances in Neurological Disorders. Understanding the burden of refractory myasthenia gravis. Available at https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6399761/. Accessed 12/20/2022.
19. American Cancer Society. Key statistics for breast cancer. Available at https://www.cancer.org/cancer/breast-cancer/about/how-common-is-breast-cancer.html. Accessed 09/22/2022.
20. National Cancer Institute. Cancer stat facts: female breast cancer subtypes. Available at https://seer.cancer.gov/statfacts/html/breast-subtypes.html. Accessed 09/22/2022.
21. Leukemia & Lymphoma Society. Mantle cell lymphoma. Available at https://www.lls.org/sites/default/files/2021-09/FS4_Mantle_Cell_Facts_0921Rev.pdf. Accessed 09/22/2022.
22. Mayo Clinic – Psoriasis. Available at https://my.clevelandclinic.org/health/diseases/6866-psoriasis. Accessed 03/15/2021.
23. National Psoriasis Foundation. About Psoriasis. Available at https://www.psoriasis.org/about-psoriasis. Accessed 03/15/2021.
24. Wu, J. Contemporary Management of Moderate to Severe Plaque Psoriasis. AJMC. Available at https://ajmc.s3.amazonaws.com/_media/_pdf/AJMC_A798_PlaquePsoriasis.pdf. Accessed 12/20/2022.
25. National Kidney Foundation – IgA nephropathy. Available at https://www.kidney.org/atoz/content/iganeph. Accessed 09/20/2022.
26. National Kidney Foundation. Available at https://www.kidney.org/news/hundreds-iga-nephropathy-patients-share-experience-fda-professionals-drug-makers. Accessed 09/20/2022.
27. Bagchi S, Mani K, Swamy A, et al. Supportive management of IgA nephropathy with renin-angiotensin blockade, the AIIMS primary IgA nephropathy cohort (APPROACH) study. Available at https://www.kireports.org/action/showPdf?pii=S2468-0249%2821%2900089-9. Accessed 09/20/2022.

*U.S. Food and Drug Administration