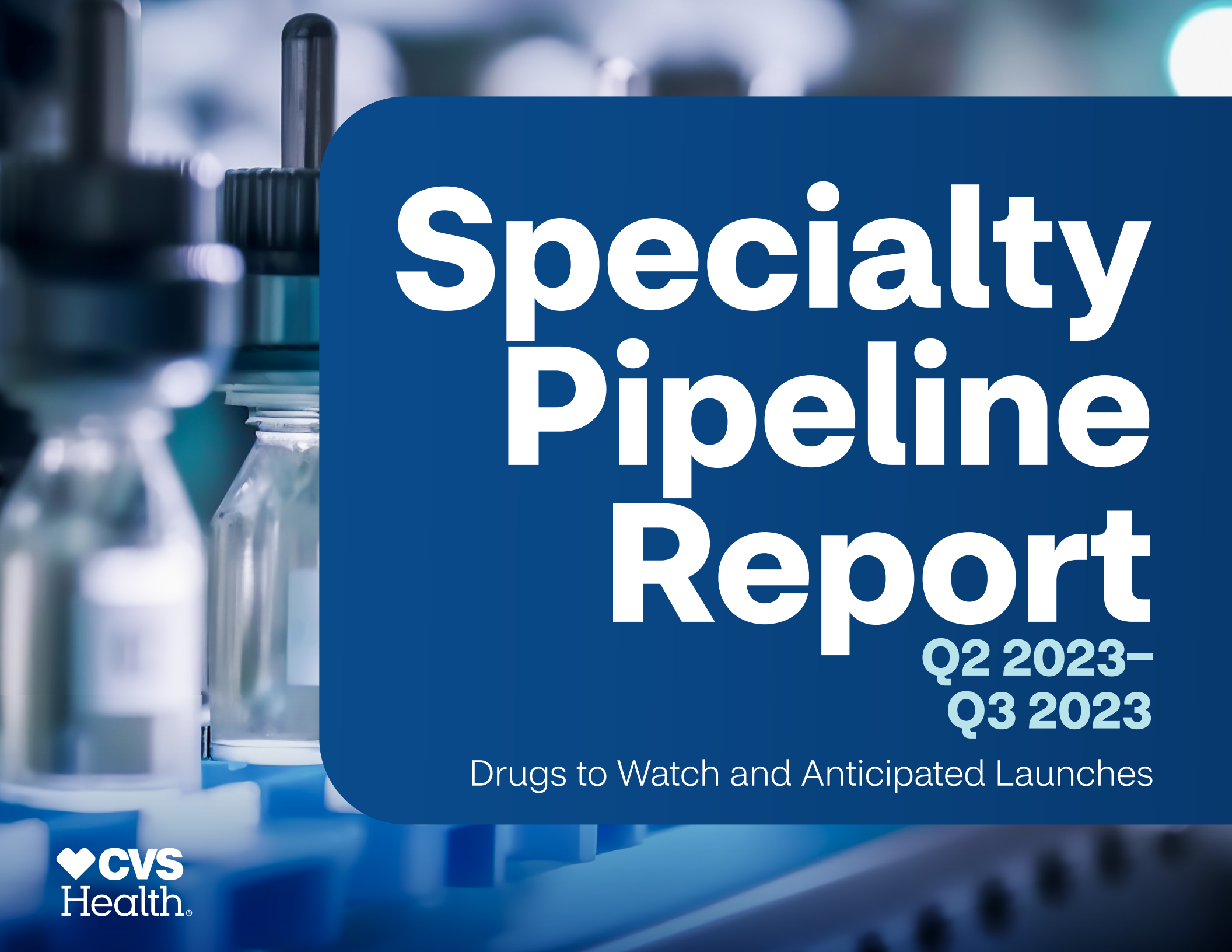

Specialty Pipeline Report

Q2 2023–Q3 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 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
Atopic Dermatitis (AD)	**lebrikizumab** SC Eli Lilly	The treatment of moderate-to-severe AD in patients aged 12 years and older	Pending FDA approval 09/20/2023	AD, also referred to as eczema, is a chronic relapsing 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 15 to 20% of children and 1 to 3% of adults.[4] Up to 40% of patients with AD have moderate-to-severe disease.[5]	**SC:** Adbry (tralokinumab-ldrm), Dupixent (dupilumab) **Oral:** Cibinqo (abrocitinib), Rinvoq (upadacitinib) Numerous topical therapies may be used.	Lebrikizumab would provide an additional SC option for moderate-to-severe disease. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit

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
Hemophilia	**concizumab** SC Novo Nordisk	The prevention of bleeding episodes in patients with hemophilia A and B with inhibitors	Pending FDA approval 04/23/2023	Hemophilia A is a genetic disorder caused by missing or defective factor VIII (FVIII) whereas hemophilia B is caused by missing or defective factor IX (FIX). People with hemophilia bleed longer than those individuals without missing or defective factors. The frequency and severity of bleeding episodes depends on the quantity of FVIII or FIX the person naturally produces. There are between 30,000 and 33,000 males with hemophilia in the U.S, with ~17,000 to 19,000 having severe disease. Hemophilia A accounts for 80% to 85% of cases while hemophilia B accounts for 15% to 20% of cases. Approximately 30% of patients with severe hemophilia A and 2 to 3% of patients with severe hemophilia B develop inhibitors to factor replacement therapies, which makes it more difficult to stop a bleeding episode.[6,7]	**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. It would provide an additional subcutaneous treatment option for hemophilia A and would be the only subcutaneously administered therapy for hemophilia B. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit
Inflammatory Bowel Disease, Psoriasis, Rheumatoid Arthritis	**ustekinumab** SC Amgen	Biosimilar of Stelara (ustekinumab). The reference product may be FDA-approved for multiple indications, and biosimilar products may or may not be approved for all indications.	Pending FDA approval 09/01/2023	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.[8]	Stelara (ustekinumab) SC Numerous other products (both approved and off-label) can be used in the management of the various autoimmune disease states (i.e., plaque psoriasis, psoriatic arthritis, Crohn's disease, and ulcerative colitis) that Stelara is used to treat.	Ustekinumab will be the first biosimilar of Stelara to launch in the U.S. At least one other Stelara biosimilar is also awaiting FDA approval in 2023. Biosimilars of Stelara will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend (potential for decreased spend), pharmacy benefit

Ustekinumab will be the first biosimilar of Stelara to launch in the United States.

Managing dosage escalation is critical to controlling costs for IBD. Read our Insights post, "**Optimizing Treatment for Inflammatory Bowel Disease**," for more on this high-spend condition.

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
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 08/15/2023	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.[9] 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.[10]	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 08/15/2023			
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.[11] 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.[12]	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

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
Neuromuscular	**efgartigimod** SC Argenx	The treatment of generalized myasthenia gravis (MG) in adults	Pending FDA approval 06/20/2023	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.[13] The prevalence of refractory MG ranges from ~10% to 20%.[14]	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
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.[15] 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.[16] Approximately 20% of patients have moderate-to-severe disease.[17]	**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

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
Respiratory Syncytial Virus (RSV)	**Beyfortus (nirsevimab)** IM AstraZeneca/ Sanofi	The prevention of RSV infection in newborns and infants entering or during their first RSV season, and for children up to 24 months of age who remain vulnerable to severe RSV disease through their second RSV season	Pending FDA approval 07/05/2023	RSV is a respiratory virus that commonly occurs in the late fall to early spring months. It is the most common cause of lower respiratory tract infections (LRTI) in infants, with almost all individuals experiencing an RSV infection by 2 years of age. Although RSV typically causes mild, cold-like symptoms, it may result in a more severe, life-threatening illness such as bronchiolitis (inflammation of the small airways in the lung) or pneumonia (infection of the lungs).[18] According to the Centers for Disease Control and Prevention, up to 80,000 children age 5 and younger are hospitalized yearly in the U.S. due to RSV.[18]	Synagis (palivizumab) IM - prevention of RSV only in high-risk premature or immunocompromised infants	Beyfortus was granted Breakthrough Therapy designation and would provide the first immunization to protect all infants regardless of risk status across the RSV season with a single dose. It will be included in Specialty Guideline Management. **Anticipated impact:** Incremental spend, pharmacy benefit
Sleep Disorders	Lumryz (sodium oxybate controlled release) oral Avadel Pharmaceuticals	The treatment of excessive daytime sleepiness (EDS) and cataplexy in patients with narcolepsy	Pending FDA approval 06/17/2023	Narcolepsy is a sleep disorder in which patients experience chronic and frequent attacks of excessive daytime sleepiness (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.[19]	**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 controlled release would provide an additional therapy option that requires less frequent dosing than Xyrem or Xywav. It will be included in Specialty Guideline Management. **Anticipated impact:** Replacement spend, pharmacy benefit

Visit our website for more insights on the active drug pipeline.

1. RxPipeline, March 2023.
2. National Alopecia Areata Foundation. Available at https://www.naaf.org/faqs. Accessed 12/19/2022.
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. AJMC. Overview of Atopic Dermatitis. Available at https://www.ajmc.com/journals/supplement/2017/atopic-dermatitis-focusing-on-the-patient-care-strategy-in-the-managed-care-setting/overview-of-atopic-dermatitis-article. Accessed 03/20/2023.
5. Asthma and Allergy Foundation of America. Atopic dermatitis is America. Available at https://www.aafa.org/media/2209/Atopic-Dermatitis-in-America-Study-Overview.pdf. Accessed 01/06/2021.
6. Centers for Disease Control and Prevention. Introduction to hemophilia care. Available at https://www.cdc.gov/ncbddd/hemophilia/course/complications/inhibitors.html. Accessed 03/20/2023.
7. Hemophilia Federation of America – Inhibitors. Available at https://www.hemophiliafed.org/home/understanding-bleeding-disorders/complications/inhibitors. Accessed 03/20/2023.
8. US Food and Drug Administration. Biosimilar and interchangeable products. Available at https://www.fda.gov/drugs/biosimilars/review-and-approval. Accessed 04/13/2023.
9. MedlinePlus. Pompe disease. Available at https://medlineplus.gov/genetics/condition/pompe-disease/#synonyms. Accessed 03/24/2022.
10. 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.
11. Fabry Disease News. Available at https://fabrydiseasenews.com/what-is-fabry-disease. Accessed 10/06/2020.
12. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/fabry-disease. Accessed 10/06/2020.
13. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/myasthenia-gravis. Accessed 06/29/2021.
14. 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.
15. Mayo Clinic – Psoriasis. Available at https://my.clevelandclinic.org/health/diseases/6866-psoriasis. Accessed 03/15/2021.
16. National Psoriasis Foundation. About Psoriasis. Available at https://www.psoriasis.org/about-psoriasis. Accessed 03/15/2021.
17. 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.
18. Centers for Disease Control and Prevention. RSV in infants and young children. Available at https://www.cdc.gov/rsv/index.html. Accessed 04/13/2023.
19. National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/narcolepsy. Accessed 06/30/2021.

*U.S. Food and Drug Administration

CVS Caremark Pipeline Services