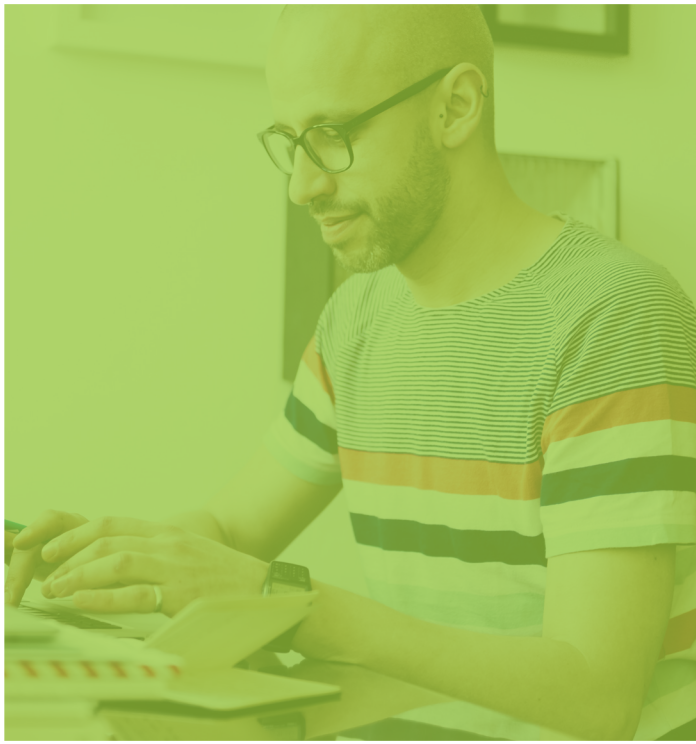
Pipeline Report

AUGUST 2023

IN THIS ISSUE:

- Recent FDA approvals for Elevidys, Roctavian, Beyfortus, and Leqembi
- Upcoming approvals for cell and gene therapies: Lifileucel, exagamglogene autotemcel, and lovotibeglogene autotemcel
- Development for important agents including donanemab, tirzepatide, and Wegovy



This quarterly publication is developed by our Clinical Pharmacy Drug Information team to increase your understanding of the drug pipeline, ensuring that you are equipped with insights to prepare for shifts in prescription drug management.

AcariaHealth™
Specialty Pharmacy

Table of Contents

Last quarter is most notable for the two gene therapies that the FDA approved within a week of each other – accelerated approval for **Elevidys** (delandistrogene moxeparvovec-rokl) for Duchenne muscular dystrophy and the long-awaited **Roctavian** (valoctocogene roxaparvovec-rvox) for severe hemophilia A. These are the first gene therapies to be approved for the conditions that they treat. Elevidys is priced at $3.2 million for a single dose, and Roctavian is priced at $2.9 million for a single dose. These are likely going to touch relatively small populations due to the rarity of the treated conditions and the confines of the labels with which each was approved. On the other end of the spectrum are the therapies that were FDA-approved during the last quarter that have much lower price points, but which have the potential to result in significant costs due to the largersize of their treatable populations, namely **Beyfortus** (nirsevimab-alip) for pediatric respiratory syncytial virus (RSV) prophylaxis in all healthy infants and **Leqembi** (lecanemab-irmb) conversion to full approval for the treatment of early Alzheimer's disease, the latter of which unlocks broader CMS coverage for people with Medicare.

In November and December 2023 we anticipate FDA potential approvals for two more gene therapies and one autologous cell therapy. The two gene therapies – **exagamglogene autotemcel** (Vertex Pharmaceuticals) and **lovotibeglogene autotemcel** (Bluebird Bio)– are proposed for the treatment of severe sickle cell disease. These two stand to dramatically change the current treatment paradigm for a condition affecting approximately 100,000 Americans, though appropriate patient selection, patient acceptance, and treatment tolerability are just a few of the mitigating factors that will need to be overcome in order for these therapies to take hold and make a significant clinical impact for the population of people with sickle cell disease. **Lifileucel** is an autologous cell therapy that is awaiting an FDA decision for its proposed indication for the treatment of advanced melanoma, and would be the first cell therapy to be approved to treat a solid tumor type.

Data readouts reported during this last quarter have bolstered the cases for FDA approval of three additional closely watched potentially significant pipeline agents. Final results from the TRAILBLAZER-ALZ 2 trial of **donanemab** for early Alzheimer's disease corroborated earlier reports from the interim analysis of slowed disease progression in donanemab-treated patients vs. placebo. Confirmation of the weight loss effects of **tirzepatide** (currently approved as Mounjaro for the treatment of type 2 diabetes) came from the SURMOUNT-3 and SURMOUNT-4 trials, which demonstrated an overall weight loss in tirzepatide-treated patients of 24.5%-26% vs. placebo, respectively. Finally, topline results from the SELECT trial of **Wegovy** (semaglutide) for cardiovascular risk reduction showed a 20% reduced risk of major adverse cardiovascular events in overweight and obese people with established cardiovascular disease and no type 2 diabetes. The FDA approval decisions on donanemab and tirzepatide are expected by the end of this year, along with an anticipated application to update the Wegovy label with the SELECT trial results.

Ross Hoffman, MD
Chief Medical Officer

To provide comments, feedback or requests for report enhancements, please email us at CPSCommunications@Centene.com.

 | Data is current as of 8/28/2023

Recent Specialty Drug Approvals

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
DERMATOLOGY					
1 2 3 **Vyjuvek** *beremagene geperpavec-svdt* topical gel	Krystal Biotech, Inc.	Dystrophic epidermolysis bullosa (DEB)	5/19/2023	· Approved for the treatment of wounds in patients ≥ 6 months of age with DEB with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene · DEB is an incurable, often fatal skin blistering condition caused by a lack of collagen in the skin due to mutations in the gene coding for type VII collagen, or COL7, a major component of the anchoring fibrils which anchor the epidermis to the underlying dermis, and provide structural adhesion in a normal individual. · The Vyjuvek label requires that it be administered by a healthcare professional, but allows for dosing either in a healthcare professional setting (e.g., clinic) or the home setting · Projected impact: new cost for a small population	~$631,000/year
4 **Litfulo** *ritlecitinib* oral capsule	Pfizer	Alopecia areata (AA)	7/7/2023	· Approved for the treatment of severe alopecia areata in adults and adolescents 12 years and older · Will compete with Olumiant for the adult population; Litfulo is the first agent to be FDA-approved for adolescent AA · Projected impact: cost replacement of existing therapy	~$49,135/year
ENDOCRINOLOGY					
5 **Elfabrio** *pegunigalsidase alfa-iwxj* intravenous infusion	Protalix BioTherapeutics	Fabry disease	5/9/2023	· Approved for the treatment of adults with confirmed Fabry disease · Is the second ERT to be FDA-approved for Fabry disease, after Fabrazyme · The Elfabrio Prescribing Information includes a Boxed Warning regarding the potential for hypersensitivity reactions including anaphylaxis · Projected impact: cost replacement of existing therapy	~$430,000/year

[1]CVS Caremark, [2]OptionCare, [3]Orsini, [4]Sonexus Pharmacy, [5]Eversana

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
A **Ngenla** *somatrogon-ghla* subcutaneous injection	Pfizer	Growth hormone deficiency	6/27/2023	· Approved for the treatment of pediatric patients aged 3 years and older who have growth failure due to inadequate secretion of endogenous growth hormone · Long-acting, once-weekly therapy · Projected impact: cost replacement of existing therapy	$99,600/year
6 **Lantidra** *donislecel-jujn* intra-hepatic portal vein infusion	CellTrans, Inc.	Type 1 diabetes (T1D)	6/28/2023	· Approved for the treatment of adults with T1D who are unable to approach target HbA1c because of current repeated episodes of severe hypoglycemia despite intensive diabetes management and education · This product consists of purified allogeneic deceased donor pancreas derived Islets of Langerhans · Projected impact: cost replacement of existing therapies	Pending launch
3 **Veopoz*** *pozelimab-bbfg* intravenous and subcutaneous injections	Regeneron	CD55 deficiency with hyperactivation of complement, angiopathic thrombosis and protein losing enteropathy (CHAPLE)	8/18/2023	· Approved for the treatment of adults and children > 1 year of age with CHAPLE disease · The Prescribing Information for Veopoz includes a Boxed Warning re: the increased risk of serious meningococcal infections · There are < 100 patients worldwide who are known to have CHAPLE and ~10 patients in the U.S. · Projected impact: new cost for a very small population	Year 1: $1.87 million Year 2 and thereafter: $1.8 million/year

[3]Orsini, [6]Hospital administration

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
HEMATOLOGY					
1 3 7 8 **Roctavian** *valoctocogene roxaparvovec-rvox* intravenous infusion	BioMarin	Hemophilia A	6/29/2023	· GENE THERAPY · Approved for the treatment of adults with severe hemophilia A without pre-existing antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test · Approximately 60% of the total hemophilia A population has severe disease · Current standard of care is factor VIII (FVIII) replacement therapy or Hemlibra · The approval of Roctavian was based on the pivotal phase 3 GENEr8-1 trial which was an open-label, single-arm phase 3 study of 134 adult patients with severe hemophilia A without inhibitors · Following a median follow-up period of 3 years, evaluable patients experienced a mean annualized bleeding rate (ABR) reduction of 52% after receiving Roctavian compared to their baseline ABR while receiving FVIII prophylaxis (2.6 bleeds/year vs 5.4 bleeds/year) · Projected impact: cost replacement of existing therapies	$2.9 million/one-time treatment

[1]CVS Caremark, [3]Orsini, [7]Accredo, [8]Optum Frontier

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
INFECTIOUS DISEASE					
15 **Beyfortus** *nirsevimab* intramuscular injection	AstraZeneca/Sanofi	Respiratory syncytial virus (RSV) prophylaxis	7/17/2023	· Approved for the prevention of RSV lower respiratory tract disease in: neonates and infants born during or entering their first RSV season; children up to 24 months of age who remain vulnerable to severe RSV disease through their second RSV season · Will compete with Synagis (palivizumab) for the high-risk infant population that requires second-season dosing; Beyfortus has a dosing advantage as a one-time intramuscular dose vs. five monthly intramuscular doses of Synagis administered throughout the RSV season · Projected impact: cost replacement of existing therapy for high-risk infants and toddlers; new cost for all other infants · On August 3, 2023, the CDC recommended that Beyfortus be covered for Medicaid-eligible infants and toddlers under the Vaccines for Children program	Infants during their first RSV season: $495/ one-time dose High-risk toddlers during their second RSV season: $990/ one-time dose
MUSCULOSKELETAL CONDITIONS					
A 3 **Elevidys** *delandistrogene moxeparvovec-rokl* intravenous infusion	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	6/22/2023	· GENE THERAPY · Accelerated approval for the treatment of ambulatory pediatric patients aged 4 through 5 years with DMD with a confirmed mutation in the DMD gene · The accelerated approval of Elevidys is based on efficacy data from two clinical trials: SRP-9001-102 and SRP-9001-103 (ENDEAVOR). The studies found increased expression of micro-dystrophin in Elevidys-treated patients and a numerical advantage over placebo in functional outcomes – the change in North Star Ambulatory Assessment (NSAA) score – in a subgroup analysis for subjects aged 4 through 5 years · The ongoing Phase 3 EMBARK confirmatory trial raises the possibility of expansion of the FDA-approved treatable population if results reported at the end of this year are positive · Projected impact: new cost in a therapeutic area that historically has had relatively low medication therapy cost, with some cost offset for the small population that is currently using exon-skipping therapies	$3.2 million/ one-time treatment

[3]Orsini, [15]Pending

 | Data is current as of 8/28/2023

CONTINUED

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
1 9 10 **Rystiggo** *rozanolixizumab-noli* subcutaneous infusion	UCB	Generalized myasthenia gravis (gMG)	6/26/2023	· Approved for the treatment of gMG in adult patients who are anti-acetylcholine receptor (AChR) or antimuscle-specific tyrosine kinase (MuSK) antibody positive · Vyvgart intravenous and Vyvgart Hytrulo subcutaneous forms, along with Soliris and Ultomiris, are also FDA-approved for AChR-positive gMG · Rystiggo is the only agent now FDA-approved for MuSK-positive gMG; ~8% of the gMG population is MuSK-positive · Projected impact: cost replacement of existing therapies	$435,000/year
1 **Sohonos** *palovarotene* oral capsules	Ipsen	Fibrodysplasia ossificans progressiva (FOP)	8/16/2023	· Approved for reduction in the volume of new heterotopic ossification in adults and children aged ≥ 8 years for females and ≥ 10 years for males with FOP · FOP is an ultra-rare genetic disorder with an estimated U.S. prevalence of 0.88/one million individuals; ~297 cases have been reported in the U.S. · Projected impact: new cost for a small population	$624,000/year

[1]CVS Caremark, [9]PANTHERx, [10]KabaFusion

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
NEUROLOGY					
[11] **Leqembi** *lecanemab-irmb* intravenous infusion	Eisai/Biogen	Alzheimer's disease (AD)	7/6/2023	· Conversion of the previously granted accelerated approval to a traditional approval for the treatment of AD: Treatment with Leqembi should be initiated in patients with mild cognitive impairment or mild dementia stage of disease, the population in which treatment was initiated in clinical trials · The approval conversion was accompanied by a new Boxed Warning in the Leqembi label re: amyloid-related imaging abnormalities (ARIA) with the suggestion to test for ApoE ε4 status to assess for potentially higher ARIA risk · With traditional approval CMS coverage of Leqembi is now broadened since enrollment in a clinical trial is no longer required · Enrollment in a patient registry is still required for CMS coverage · Projected impact: higher utilization due to broader CMS coverage for people with Medicare, though general concerns persist regarding Leqembi's safety and overall benefit profile, along with its cumbersome screening and ARIA monitoring requirements	$26,500/year
ONCOLOGY					
[12] **Epkinly** *epcoritamab-bysp* subcutaneous injection	Genmab/AbbVie	Large B-cell lymphoma (LBCL)	5/19/2023	· Approved for the treatment of adults with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), not otherwise specified, including DLBCL arising from indolent lymphoma, and high-grade B-cell lymphoma after ≥ 2 lines of systemic therapy · The Epkinly Prescribing Information includes a boxed warning regarding cytokine release syndrome and immune effector cell-associated neurotoxicity syndrome · Potential competitor to CAR T-cell therapies and Polivy, as third-line therapy for LBCL · Projected impact: cost replacement of existing therapies	$337,500/ average 9-month duration of therapy

[11]Soleo Health, [12]Doctors office or infusion center administration

 | Data is current as of 8/28/2023

CONTINUED

Available at: A AcariaHealth # Other

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
6 12 **Columvi** *glofitamab-gxbm* intravenous infusion	Roche	Large B-cell lymphoma (LBCL)	6/15/2023	· Approved for the treatment of adult patients with relapsed or refractory DLBCL, not otherwise specified or LBCL arising from follicular lymphoma, after two or more lines of systemic therapy · The Columvi Prescribing Information includes a Boxed Warning re: cytokine release syndrome · Potential competitor to CAR T-cell therapies, Epkinly, and Polivy, as third-line therapy for LBCL · Projected impact: cost replacement of existing therapies	$350,000/ 12-cycle course of therapy
13 14 **Vanflyta** *quizartinib* oral tablet	Daiichi Sankyo	Acute myeloid leukemia (AML)	7/20/2023	· Approved for use in combination with standard cytarabine and anthracycline induction and cytarabine consolidation, and as maintenance monotherapy following consolidation chemotherapy, for the treatment of adult patients with newly diagnosed AML that is FLT3-ITP-positive as detected by an FDA-approved test · FDA-approved with a Boxed Warning re: increased risk of QT prolongation, torsades de pointes, and cardiac arrest · Rydapt (midostaurin) is also FDA-approved for the treatment of newly diagnosed FLT3-mutated AML · Projected impact: cost replacement of existing therapies	$199,290/year
6 12 **Talvey** *talquetamab-tgvs* subcutaneous infusion	Janssen	Relapsed or refractory multiple myeloma (RRMM)	8/9/2023	· FDA granted accelerated approval for the treatment of adult patients with relapsed or refractory multiple myeloma, who have previously received at least four prior lines of therapy, including a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 antibody · Initial doses are administered in an inpatient setting, with outpatient maintenance dosing · Tecvayli, Elrexfio, Abecma, and Carvykti are other therapies that are FDA-approved for the same indicationProjected impact: cost replacement of existing therapies	$360,000/ 8-month average duration of therapy

[6]Hospital administration, [12]Doctor's office or infusion center administration, [13]Biologics, [14]Onco360

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC)
[15] **Akeega** *niraparib + abiraterone* oral tablets	Janssen	Prostate cancer	8/11/2023	· Approved for use in combination with prednisone for the treatment of adult patients with deleterious or suspected deleterious BRCA-mutated metastatic castration-resistant prostate cancer (mCRPC) · Niraparib without abiraterone is also FDA-approved as Zejula for the treatment of ovarian, fallopian tube, and primary peritoneal cancers · Lynparza and Rubraca are two other agents with the same mechanism of action that are also FDA-approved for BRCA-mutated mCRPC · Projected impact: cost replacement of existing therapies	$225,000/year
[6] [12] **Elrexfio** *elranatamab-bcmm* subcutaneous injection	Pfizer	Relapsed or refractory multiple myeloma (RRMM)	8/14/2023	· FDA granted accelerated approval for the treatment of adult patients with relapsed or refractory multiple myeloma, who have previously received at least four prior lines of therapy, including a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 antibody · Initial doses are administered in an inpatient setting, with outpatient maintenance dosing · Tecvayli, Talvey, Abecma, and Carvykti are other therapies that are FDA-approved for the same indication · Projected impact: cost replacement of existing therapies	$330,000/ 8-month average duration of therapy
OPHTHALMOLOGY					
[7] **Izervay** *avacincaptad pegol* intravitreal injection	Iveric bio, Inc.	Geographic atrophy (GA)	8/4/2023	· Approved for the treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) · Syfovre (pegcetacoplan) has a similar mechanism of action and is FDA-approved for the same indication · Projected impact: cost replacement of existing therapies	$2,100/single-dose vial

[6]Hospital administration, [7] Accredo, [12]Doctor's office or infusion center administration, [15]Pending

 | Data is current as of 8/28/2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
COAGULATION DISORDERS						
PF-06838435/SPK-9001* *fidanacogene elaparvovec* intravenous infusion	Pfizer/Spark	Hemophilia B	Gene therapy	· Current standard of care is factor IX (FIX) replacement therapy · The Phase III BENEGENE-2 study in adult males with moderately severe to severe hemophilia B met its primary endpoint of reduction in ABR of total bleeds, with a mean ABR reduction of 71% (p<0.0001) · Also reported was a 92% reduction in annualized FIX infusion rate (p<0.0001) · Mean FIX activity was 27% at 15 months and 25% at 24 months · Fidanacogene elaparvovec was generally well-tolerated; no deaths, serious adverse events associated with infusion reactions, thrombotic events, or FIX inhibitors were reported · Would compete with Hemgenix (etranacogene dezaparvovec-drlb) gene therapy for the same indication · The FDA accepted the BLA for review	$2-3 million/ one-time treatment	2Q 2024

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
SPK-8011* intravenous infusion	Spark/Roche	Hemophilia A	Gene therapy	· Proposed for the treatment of adults with severe disease (~60% of the total hemophilia A population) · Current standard of care is factor VIII replacement therapy or Hemlibra · In the ongoing Phase I/II trial, factor VIII expression was sustained in 21 of 23 (91%) participants with up to five years of follow-up · Of these 21 participants, there was a 92% reduction in ABR · There were no deaths, no thrombotic events, and no FVIII inhibitor development reported in the five years · The Phase III Keystone-1 trial has launched · Would compete with Roctavian (valoctocogene roxaparvovec-rvox) gene therapy for the same indication	$2-3 million per one-time treatment	2024

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
SB-525* *giroctocogene fitelparvovec* intravenous infusion	Sangamo BioSciences, Inc/ Pfizer	Hemophilia A	Gene therapy	· For the treatment of adults with severe disease (~60% of the total hemophilia A population · Current standard of care is factor VIII replacement therapy or Hemlibra · SB-525 was being studied in the Phase III AFFINE trial, which had been voluntarily paused by the manufacturers to address the observation that some patients had factor VIII activity of 150% or more, potentially raising their risk of blood clots · After a study protocol amendment, the AFFINE trial was re-started and a pivotal readout is expected in the first half of 2024 · Meanwhile, updated results from the Phase I/II Alta trial showed that, among five patients receiving the highest dose of SB-525, mean factor VIII levels were 25.4% at two years; during Year 2, one patient had eight bleeds, while another had one · Would compete with Roctavian (valoctocogene roxaparvovec-rvox) gene therapy for the same indication	$2-3 million/ one-time treatment	2025
DERMATOLOGY						
Bimzelx *bimekizumab* subcutaneous injection	UCB	Plaque psoriasis	IL-17A and IL-17F inhibitor	· Proposed for the treatment of adults with moderate-to-severe chronic plaque psoriasis · Would compete with multiple other injectable biologic agents approved for the treatment of plaque psoriasis	$40,000/ year	3Q 2023
RG-3637 *lebrikizumab* subcutaneous injection	Eli Lilly	Atopic dermatitis	Anti-IL-13 monoclonal antibody	· Proposed for the treatment of persistent moderate-to-severe atopic dermatitis · Would compete with other biologic agents for atopic dermatitis, such as Adbry and Dupixent	$40,000/ year	9/20/2023

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ENDOCRINOLOGY						
DCR-PHXC* *nedosiran* subcutaneous injection	Dicerna	Primary hyperoxaluria type 1 (PH1)	RNA interference therapy	· Proposed for the treatment of PH1 in patients ≥ 6 years of age · Would compete with Oxlumo for the same indication	$500,000/ year	9/1/2023
AT-GAA* *cipaglucosidase alfa/ miglustat* intravenous infusion plus oral therapy	Amicus Therapeutics	Pompe disease	Cipaglucosidase alfa: recombinant human acid alpha -glucosidase enzyme replacement therapy (ERT) Miglustat: pharmacological chaperone	· Proposed for the treatment of late-onset Pompe disease · Estimated U.S prevalence of Pompe disease: ~1 in 40,000 people · Lumizyme and Nexviazyme are available FDA approved ERT alternatives (Nexviazyme for late-onset disease) · In October 2022, the FDA issued a Deferred Action Letter due to the inability to conduct a required inspection of a manufacturing site due to COVID-19-related travel restrictions · The manufacturer continues to expect the FDA to approve the two components of AT-GAA, including the BLA for cipaglucosidase alfa and the NDA for miglustat, together	$500,000/ year	3Q 2023
TAK-755* *apadamtase alfa* intravenous infusion	Takeda	Thrombotic thrombocytopenic purpura	Enzyme replacement therapy	· Proposed for the treatment of congenital thrombotic thrombocytopenic purpura (cTTP) · cTTP is an ultra-rare, chronic subtype of TTP that has an estimated prevalence of fewer than one case/ million · The current standard of care for cTTP is plasma therapy	$500,000/ year	4Q 2023

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date

OTL-200*
atidarsagene autotemcel
intravenous infusion
	Orchard Therapeutics	Metachromatic leukodystrophy (MLD)	Gene therapy	• MLD is a rare and life-threatening inherited disease occurring in approximately one in every 100,000 live births, caused by a mutation in the ARSA gene
				  • In its late infantile form, mortality at 5 years from onset is estimated at 50% and 44% at 10 years for juvenile patients
				• Currently, there are no effective treatments for MLD
				• Rolling BLA submission for Priority Review is completed
				• The Institute for Clinical and Economic Review (ICER) released a Draft Evidence Report assessing the comparative clinical effectiveness and value of OTL-200 for MLD
				  • In the Draft report, ICER assigned an Evidence Rating of high certainty of "A" (substantial net health benefit) vs. usual standard of care for OTL-200 treatment in children with presymptomatic late-infantile and early juvenile forms of MLD
				  • An Evidence Rating of moderate certainty of a "C++" (comparable, small or substantial net health benefit with high certainty of at least a comparable net health benefit) vs. usual standard of care was assigned for early symptomatic early juvenile MLD
					$3-4 million/ one-time treatment	2024

GASTROENTEROLOGY						
APD334 *etrasimod* oral therapy	Arena Pharmaceuticals	Ulcerative colitis (UC)	G-protein coupled receptor and sphingosine 1 phosphate type 1 modulator	• Proposed for the treatment of moderate-to-severe UC • Would compete with Zeposia as well as other available agents for UC	$95,000/year	2H 2023

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
HEMATOLOGY						
Aphexda *motixafortide* subcutaneous injection	BioLineRx Ltd	Bone marrow transplant	CXCR4 chemokine receptor inhibitor	· Proposed for stem cell mobilization for autologous bone marrow transplantation in multiple myeloma patients · Could compete with Mozobil (plerixafor) subcutaneous injections	$30,000/ course of therapy	9/9/2023
CTX001* *exagamglogene autotemcel* intravenous infusion	CRISPR Therapeutics/ Vertex	Sickle cell disease (SCD); transfusion-dependent beta-thalassemia (TDT)	CRISPR/Cas9 gene-editing therapy	· Proposed for the treatment of severe SCD in patients aged ≥ 12 years and older and for the treatment of TDT in patients aged ≥ 12 years · SCD affects approximately 100,000 Americans · In the Phase I/II/III CLIMB-121 trial, all 31 treated patients with severe SCD characterized by recurrent vaso-occlusive events (VOEs) were free of VOEs after CTX001 infusion through duration of follow-up, up to 32.3 months · Could be the first gene therapy approved for the treatment of SCD · Would compete with Zynteglo (betibeglogene autotemcel) gene therapy for the TDT indication · The FDA has accepted the BLA submissions for both indications	$2.5 million/ one-time treatment	SCD: 12/8/2023 TDT: 3/30/2024
bb1111* *lovotibeglogene autotemcel* intravenous infusion	bluebird bio	SCD	Lentiviral vector-based gene therapy	· Proposed for the treatment of SCD in patients ≥ 12 years of age who have a history of vaso-occlusive events · SCD affects approximately 100,000 Americans · Updated data from the Phase I/II HGB-206 study showed that 31 of 32 (96%) patients treated with bb1111 experienced complete resolution of severe VOEs through 24 months of follow-up · The FDA has accepted the BLA for review	$2.5 million/ one-time treatment	12/20/2023

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
MUSCULOSKELETAL CONDITIONS						
vamorolone oral therapy	Santhera	DMD	Steroidal anti-inflammatory agent	· Would compete with generic steroids and Emflaza	$150,000/ year	10/26/2023
givinostat oral suspension	Italfarmaco	DMD	Histone deacetylase (HDAC) inhibitor	· Twice-daily oral dosing, for use as an adjunct to existing corticosteroid therapy · Acts on the pathogenetic events downstream of DMD-related genetic defects, thus is potentially a treatment for the whole DMD population	$350,000/ year	12/21/2023
PF-06939926* *fordadistrogene movaparvovec* intravenous infusion	Pfizer	DMD	Gene therapy	· One-time treatment · Three serious adverse effects were identified in the Phase III CIFFREO trial, muscle weakness including two cases of myocarditis, attributed to the gene therapy · The study protocol was amended to exclude patients with any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting exons 9-13, inclusive, or a deletion that affects both exon 29 and exon 30; these mutations are estimated to represent ~15% of patients with DMD · There are indications that the muscle-related adverse effects associated with specific exon gene mutations may be a class effect across DMD gene therapies · Phase 3 data is anticipated during 1H 2024	$2-3 million/ one-time treatment	2025

Expected to cost ≥ $500,000 per member.

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
GALGT2* *AAVrh74.MHCK.GALGT2* intra-arterial injection	Sarepta Therapeutics	DMD	Gene therapy	· Would compete with SRP-9001 gene therapy for those with mutations between exons 18-58 · SRP-9001 is further along in the pipeline process, but comparative safety and efficacy are undetermined	$2-3 million/ one-time treatment	2025
NEUROLOGY						
RA101495 *zilucoplan* subcutaneous injection	Ra Pharmaceuticals	Generalized myasthenia gravis (gMG)	Complement inhibitor	· Proposed for the treatment of gMG in adult patients who are acetylcholine receptor antibody positive · Daily self-administered subcutaneous injection · Other complement inhibitors that are also FDA-approved for gMG include Soliris and Ultomiris, both of which are infused intravenously for gMG · Additional competing therapies for gMG include Vyvgart, Vyvgart Hytrulo, Rystiggo, and immunoglobulin therapy	$450,000/ year	10/1/2023
IONIS-TTR-LRx *eplontersen* subcutaneous injection	Ionis Pharmaceuticals	Polyneuropathy	Ligand-conjugated antisense agent	· Proposed for the treatment of hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN) · Injected subcutaneously once every four weeks · Onpattro, Tegsedi, and Amvuttra are all FDA-approved for the same indication; eplontersen may have a better safety profile than weekly SC Tegsedi, against which it was compared in the Neuro-TTRansform study	$475,000/ year	12/22/2023

Expected to cost ≥ $500,000 per member.

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date

LY3002813
donanemab
intravenous infusion
	Eli Lilly	Early AD	Anti-amyloid-beta monoclonal antibody	• Proposed for the treatment of early symptomatic AD
				• Final results of the Phase III TRAILBLAZER-2 trial for donanemab for early Alzheimer's disease were reported
				  • The primary endpoint of change from baseline until 18 months on the integrated Alzheimer's Disease Rating Scale (iADRS) was met, with a slowing of clinical decline of 35% at 18 months compared to placebo in patients with intermediate brain protein tau levels
				  • In a combined group of patients with intermediate and high tau levels, there was a 29% slowing of clinical decline on the Clinical Dementia Rating-Sum of Boxes score (CDR-SB) in donanemab-treated patients vs. those who received placebo
				  • Rates of amyloid-related imaging abnormalities (ARIA) were approximately double of what were previously observed in the Leqembi trials
				  • In this study, the incidence of serious ARIA was 1.6%, including two participants whose death was attributed to ARIA and a third participant who died after an incident of serious ARIA
				• Donanemab and Leqembi have not been directly compared in any head-to-head trials
				• A BLA submission requesting full approval has occurred, with FDA approval anticipated by the end of the year; if approved, donanemab would compete with Leqembi, and it would be subject to the same coverage restrictions imposed by the CMS NCD for this class of agents
					$30,000/ year	4Q 2023

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date

PTC-AADC* *eladocagene exuparvovec* intraputamenal injection	PTC Therapeutics	Aromatic L-amino acid decarboxylase (AADC) deficiency	Gene therapy	• There are no approved therapies for the treatment of AADC deficiency, which is an ultra-rare enzyme deficiency disorder
				• Estimated prevalence: ~5,000 patients worldwide, with a live-birth incidence of approximately 1 in 40,000 worldwide
				• Five-year follow-up results from a clinical trial show that motor function improvements after PTC-AADC therapy were sustained, demonstrating that the treatment effect is durable
				  • Across three clinical trials, improvements in motor development were recorded in all children from as early as three months
				  • Cognitive and language skills were also reported to improve significantly from baseline, as measured by Bayley-III scores, with children able to understand their caregivers and express themselves
				  • The rate of respiratory infection declined from an average of 2.4 episodes/year at 12 months to 0.6 episodes/year at two years and 0.3 episodes/year at five years
				  • Almost all treated children went from a baseline weight below the third percentile to making age-appropriate weight gains by 12 months following treatment
				• Planned BLA submission in 3Q 2023
					$3-4 million/ one-time treatment	2024

***Expected to cost ≥ $500,000 per member.**

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
NurOwn *debamestrocel* intrathecal injection	BrainStorm Cell Therapeutics	Amyotrophic lateral sclerosis (ALS)	Autologous mesenchymal stem cell therapy	• One-time course of therapy consisting of 3 intrathecal injections at weeks 0, 8, and 16 • Mesenchymal stem cells are harvested from each person with ALS, expanded, and engineered to secrete neurotrophic factors (NTFs), before being reinfused into the patient. These cells deliver multiple NTFs and immunomodulatory cytokines directly to the site of damage to attempt to slow or stabilize disease progression	$350,000/ one-time course of therapy	12/8/2023
ONCOLOGY						
CYT387 *momelotinib* oral capsule	Sierra Oncology	Myelofibrosis	Selective ACVR1/ ALK2, JAK1, JAK2 inhibitor	• Proposed for the treatment of patients with myelofibrosis who have previously received a JAK inhibitor	$197,000/ year	9/16/2023
JS001 *toripalimab* intravenous infusion	Junshi Biosciences/ Coherus	Nasopharyngeal carcinoma (NPC)	Anti-PD-1 monoclonal antibody	• Proposed for use in combination with gemcitabine and cisplatin for first-line treatment for patients with advanced recurrent or metastatic NPC and as monotherapy for second-line or above treatment of recurrent or metastatic NPC after platinum-containing chemotherapy • About 80% of people with NPC have entered the middle and advanced stage when clinically diagnosed, with lymph node metastasis or distant metastasis. After development of distant metastasis, the 5-year survival rate is less than 50% • FDA conducted the required facility inspection with 3 flagged observations. Coherus plans to submit its response to the FDA on these observations	$200,000/ year	3Q 2023

 | Data is current as of 8/28/2023

CONTINUED

AcariaHealth Specialty Pharmacy

Pipeline Report

August 2023

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
BGB-A317 *tislelizumab* intravenous infusion	BeiGene/Novartis	Esophageal carcinoma	Humanized IgG4 anti-PD-1 monoclonal antibody	· Proposed for the treatment of patients with unresectable recurrent locally advanced or metastatic esophageal squamous cell carcinoma after prior systemic therapy · The FDA deferred its decision on the BLA due to lack of inspections in a foreign manufacturing facility as a result of COVID-19-related travel restrictions	$175,000/ year	3Q 2023
LN-144* *lifileucel* intravenous injection	Iovance	Melanoma	Tumor infiltrating lymphocyte cell therapy	· Proposed for the treatment of advanced (unresectable or metastatic) melanoma that has progressed after anti-PD-1/L1 therapy, and if BRAF mutation positive, also prior BRAF or BRAF/MEK inhibitor therapy · Administered as a one-time dose · If approved, this would be the first cell therapy to target a solid tumor	$500,000/ one-time treatment	11/25/2023
repotrectinib oral therapy	Bristol Myers Squibb	Non-small cell lung cancer (NSCLC)	Tyrosine kinase inhibitor	· Proposed for the treatment of patients with ROS1-positive locally advanced or metastatic NSCLC	$200,000/ year	11/27/2023
nirogacestat oral tablet	SpringWorks Therapeutics	Desmoid tumors	Amyloid precursor protein secretase inhibitor	· Proposed for the treatment of adults with desmoid tumors · There are currently no FDA-approved therapies for the treatment of desmoid tumors; radiation therapy and off-label chemotherapy are current treatment options	$200,000/ year	11/27/2023
HMPL-013 *fruquintinib* oral therapy	Takeda	Colorectal cancer	VEGF receptor inhibitor	· Proposed for the treatment of patients with metastatic colorectal cancer who have been previously treated with fluoropyrimidine-, oxaliplatin-, and irinotecan-based chemotherapy, an anti-VEGF biological therapy, and, if RAS wild-type, an anti-epidermal growth factor receptor therapy	$200,000/ year	11/30/2023

Expected to cost ≥ $500,000 per member.

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
AZD5363 *capivasertib* oral tablet	AstraZeneca	Breast cancer	Adenosine triphosphate competitive inhibitor	• Proposed for use in combination with fulvestrant, for the treatment of patients with hormone receptor–positive, HER2-negative, locally advanced or metastatic breast cancer following recurrence or progression on or after endocrine-based therapy	$200,000/ year	12/12/2023
Abecma *idecabtagene vicleucel* intravenous infusion	Bristol Myers Squibb	Relapsed or refractory multiple myeloma (RRMM)	CART-cell therapy	• **NEW INDICATION FOR AN EXISTING CAR T-CELL THERAPY** • Proposed for the treatment of adult patients with RRMM who have received an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody • Proposes use as third line of therapy or later; Abecma is currently FDA-approved for use after four or more prior lines of therapy	$458,000 one time	12/16/2023
cosibelimab intravenous infusion	Checkpoint Therapeutics	Cutaneous squamous cell carcinoma (cSCC)	Anti-PD-L1 antibody	• Proposed for the treatment of patients with metastatic cSCC or locally advanced cSCC who are not candidates for curative surgery or radiation	$180,000/ year	1/3/2024
IMAB362 *zolbetuximab* intravenous infusion	Astellas	Gastric or gastroesophageal junction (GEJ) adenocarcinoma	Anti-claudin 18.2 (CLDN18.2) monoclonal antibody	• Proposed for first-line treatment of patients with locally advanced unresectable or metastatic HER2-negative gastric or GEJ adenocarcinoma whose tumors are CLDN18.2-positive	$200,000/ year	1/12/2024
rivoceranib oral therapy	Elevar Therapeutics	Hepatocellular carcinoma	Tyrosine kinase inhibitor	• Proposed for use in combination with camrelizumab as first-line treatment option for unresectable hepatocellular carcinoma • Camrelizumab is a PD-1 inhibitor	$200,000/ year	5/16/2024

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
OPHTHALMOLOGY						
Lytenava *bevacizumab-vikg* intraocular injection	Outlook Therapeutics	Age-related macular degeneration (AMD)	Vascular endothelial growth factor (VEGF) inhibitor	• Proposed for the treatment of wet AMD • Would be the first FDA-approved ophthalmic formulation of bevacizumab to treat retinal diseases; bevacizumab is currently approved in an IV formulation for the treatment of cancer indications • Currently existing forms of IV bevacizumab are compounded into an ophthalmic form for use in ophthalmic indications • Lytenava would compete with multiple other existing ophthalmic VEGF inhibitors (e.g., Beovu, Eylea, Lucentis, Vabysmo) for this indication	$15,000/year	8/29/2023

 | Data is current as of 8/28/2023

Drug Name	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
ENDOCRINOLOGY						
denosumab subcutaneous injection	Sandoz	Prolia and Xgeva	Osteoporosis, skeletal-related complications of bone metastases, giant cell tumor of the bone, hypercalcemia of malignancy	BLA is under FDA review (BsUFA date: 12/5/2023)	No	· Would be the first approved Prolia/Xgeva biosimilar product
HEMATOLOGY						
ABP-959 *eculizumab* intravenous infusion	Amgen	Soliris	Paroxysmal nocturnal hemoglobinuria	BLA is under FDA review (BsUFA date: 2/15/2024)	No	· Would be the first approved Soliris biosimilar product · Anticipated launch: March 2025 due to a Soliris patent litigation agreement
IMMUNOLOGY						
Yuflyma *adalimumab-aaty* subcutaneous injection	Celltrion	Humira	RA, pJIA, PsA, AS, CD, UC, plaque psoriasis, hidradenitis suppurativa	FDA approval: 5/23/2023	Yes	· Citrate-free and 100 mg/mL high concentration formulation · Market launch: July 2, 2023
Abrilada, interchangeability status *adalimumab-afzb* subcutaneous injection	Pfizer	Humira	RA	BLA is under FDA review (BsUFA date: 9/15/2023)	Yes	· Previously approved biosimilar agent; currently proposed for interchangeability with Humira · Citrate-free formulation
MSB11456 *tocilizumab* subcutaneous injection and intravenous infusion	Fresenius Kabi	Actemra	RA, giant cell arteritis (GCA), pJIA, and systemic juvenile idiopathic arthritis (sJIA)	BLA is under FDA review (BsUFA date: 3Q 2023)	No	· Could be the first approved Actemra biosimilar product

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
BIIB800 *tocilizumab* intravenous infusion	Biogen	Actemra	RA, GCA, pJIA, and sJIA	BLA is under FDA review (BsUFA date: 3Q 2023)	No	• Could be the first approved Actemra biosimilar product
AVT04 *ustekinumab* subcutaneous injection	Alvotech/Teva	Stelara	Plaque psoriasis	BLA is under FDA review (BsUFA date: 10/11/2023)	No	• Could be the first approved Stelara biosimilar product • Alvotech/Teva have secured a settlement with Janssen (owns reference product Stelara) that allows a launch date for AVT04 in the U.S. no later than 2/21/2025
ABP 654 *ustekinumab* subcutaneous injection	Amgen	Stelara	Plaque psoriasis	BLA is under FDA review (BsUFA date: 2H 2023)	No	• Could be the first approved Stelara biosimilar product • Amgen has secured a settlement with Janssen that allows a launch date for ABP 654 in the U.S. no later than 1/1/2025

 | Data is current as of 8/28/2023

CONTINUED

Drug Name	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
NEUROLOGY						
Tyruko *natalizumab-sztn* intravenous infusion	Sandoz	Tysabri	Relapsing forms of multiple sclerosis; Crohn's disease	FDA approval: 8/24/2023	No	· FDA-approved for the treatment of adults with relapsing forms of multiple sclerosis, and for inducing and maintaining clinical response and remission in adults with moderately to severely active Crohn's disease who also have evidence of inflammation and have had an inadequate response or inability to tolerate conventional therapies and inhibitors of tumor necrosis factor · Is the first approved Tysabri biosimilar product
ONCOLOGY						
HLX02 *trastuzumab* intravenous infusion	Henlius Biotech	Herceptin	Breast cancer, gastric or GEJ cancer	BLA is under FDA review (BsUFA date: 12/15/2023)	Yes	· Would be the sixth Herceptin biosimilar to be FDA-approved after Herzuma, Kanjinti, Ogivri, Ontruzant, and Trazimera
DRL_RI *rituximab* intravenous infusion	Dr. Reddy's Laboratories	Rituxan	Non-Hodgkin's lymphoma, RA	BLA is under FDA review (BsUFA date: 2Q 2024)	Yes	· Would be the fourth Rituxan biosimilar to be FDA-approved after Riabni, Ruxience, and Truxima
OPHTHALMOLOGY						
Xlucane *ranibizumab* intraocular injection	Xbrane Biopharma/ Bausch & Lomb	Lucentis	Wet AMD	BLA is under FDA review (BsUFA date: 4/21/2024)	Yes	· Would be the third Lucentis biosimilar to be FDA-approved after Byooviz and Cimerli

 | Data is current as of 8/28/2023

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
gefitinib	Iressa	Qilu Pharmaceutical Co.	5/3/2023
darunavir ethanolate	Prezista (400 mg, 600 mg, 800 mg tablets)	Lupin	6/1/2023
plerixafor	Mozobil	Fresenius	7/11/2023
Pipeline Agents			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**ANTICIPATED LAUNCH DATE**
riluzole	Tiglutik Kit	Not yet determined	4Q 2023
bendamustine hydrochloride	Treanda (solution)	Fresenius	2H 2023
prednisone	Rayos	Actavis/Teva	2023
nilotinib hydrochloride	Tasigna	Apotex	1/5/2024
raltegravir potassium	Isentress (400 mg tablet)	Hetero	4/4/2024
midostaurin	Rydapt	Lupin	10/28/2024

Includes generic agents with ≥ 50% launch probability

 | Data is current as of 8/28/2023

Term	Definition
AA	alopecia areata
AADC	aromatic L-amino acid decarboxylase
AAV5	adeno-associated virus serotype 5
ABR	annualized bleeding rate
AChR	acetylcholine receptor
AD	Alzheimer's disease
aGvHD	acute graft vs. host disease
ALS	amyotrophic lateral sclerosis
AMD	age-related macular degeneration
AML	acute myeloid leukemia
ARIA	amyloid-related imaging abnormalities
AS	ankylosing spondylitis
ATTRv-PN	transthyretin-mediated amyloid polyneuropathy
BLA	biologics license application

Term	Definition
BsUFA	Biosimilar User Fee Act
CAR T-cell	chimeric antigen receptor T-cell
CD	Crohn's disease
CDR-SB	Clinical Dementia Rating-Sum of Boxes
CHAPLE	CD55 deficiency with hyperactivation of complement, angiopathic thrombosis and protein losing enteropathy
CLDN18.2	claudin 18.2
COL7A1	collagen type VIII alpha 1 chain
COVID-19	coronavirus disease 2019
cSCC	cutaneous squamous cell carcinoma
cTTP	congenital thrombotic thrombocytope-nic purpura
CV	cardiovascular
DEB	dystrophic epidermolysis bullosa
DED	dry eye disease
DLBCL	diffuse large B-cell lymphoma

Term	Definition
DMD	Duchenne muscular dystrophy
ERT	enzyme replacement therapy
FDA	Food and Drug Administration
FIX	factor IX
FL	follicular lymphoma
FOP	fibrodysplasia ossificans progressiva
FVIII	factor VIII
GA	geographic atrophy
GCA	giant cell arteritis
GEJ	gastroesophageal junction
GIP	glucose-dependent insulinotropic polypeptide
GLP-1	glucagon-like peptide-1
gMG	generalized myasthenia gravis
HCC	hepatocellular carcinoma

 | Data is current as of 8/28/2023

CONTINUED

Term	Definition
HDAC	histone deacetylase
HF	heart failure
ICER	Institute for Clinical and Economic Review
LBCL	large B-cell lymphoma
MA-LRTI	medically attended lower respiratory tract illness
mCRPC	metastatic castration-resistant prostate cancer
MDD	major depressive disorder
MLD	metachromatic leukodystrophy
MS	multiple sclerosis
MuSK	muscle-specific tyrosine kinase
NPC	nasopharyngeal carcinoma
NSAA	North Star Ambulatory Assessment
NSCLC	non-small cell lung cancer
NTF	neurotrophic factor

Term	Definition
PD-L1	programmed death-ligand 1
PH1	primary hyperoxaluria type 1
pJIA	polyarticular juvenile idiopathic arthritis
PPD	post-partum depression
PsA	psoriatic arthritis
RA	rheumatoid arthritis
RAR-γ	retinoic acid receptor gamma
RRMM	relapsed or refractory multiple myeloma
RSV	respiratory syncytial virus
SCD	sickle cell disease
SGLT2	sodium-glucose co-transporter 2
sJIA	systemic juvenile idiopathic arthritis
T1D	type 1 diabetes
T2DM	type 2 diabetes mellitus

Term	Definition
TDT	transfusion-dependent beta-thalassemia
UC	ulcerative colitis
VEGF	vascular endothelial growth factor
VOE	vaso-occlusive event
WAC	Wholesale Acquisition Cost
VOE	vaso-occlusive event
WAC	Wholesale Acquisition Cost

 | Data is current as of 8/28/2023

AcariaHealth is a national comprehensive specialty pharmacy focused on improving care and outcomes for patients living with complex conditions, such as hepatitis C, multiple sclerosis, oncology, rheumatoid arthritis, hemophilia, cystic fibrosis and other conditions. Offering specialized care management services in these disease states, AcariaHealth is dedicated to enhancing the patient care offering, collaborating with providers and capturing relevant data to measure patient outcomes.

Learn more about how we put patients first while providing exceptional specialty pharmacy care at AcariaHealth.com. You can also connect with us on LinkedIn to view our latest news and updates.

AcariaHealth.com

Linkedin.com/company/acariahealth-inc.

8517 Southpark Circle, Suite 200
Orlando, FL 32819

AcariaHealth™
Specialty Pharmacy