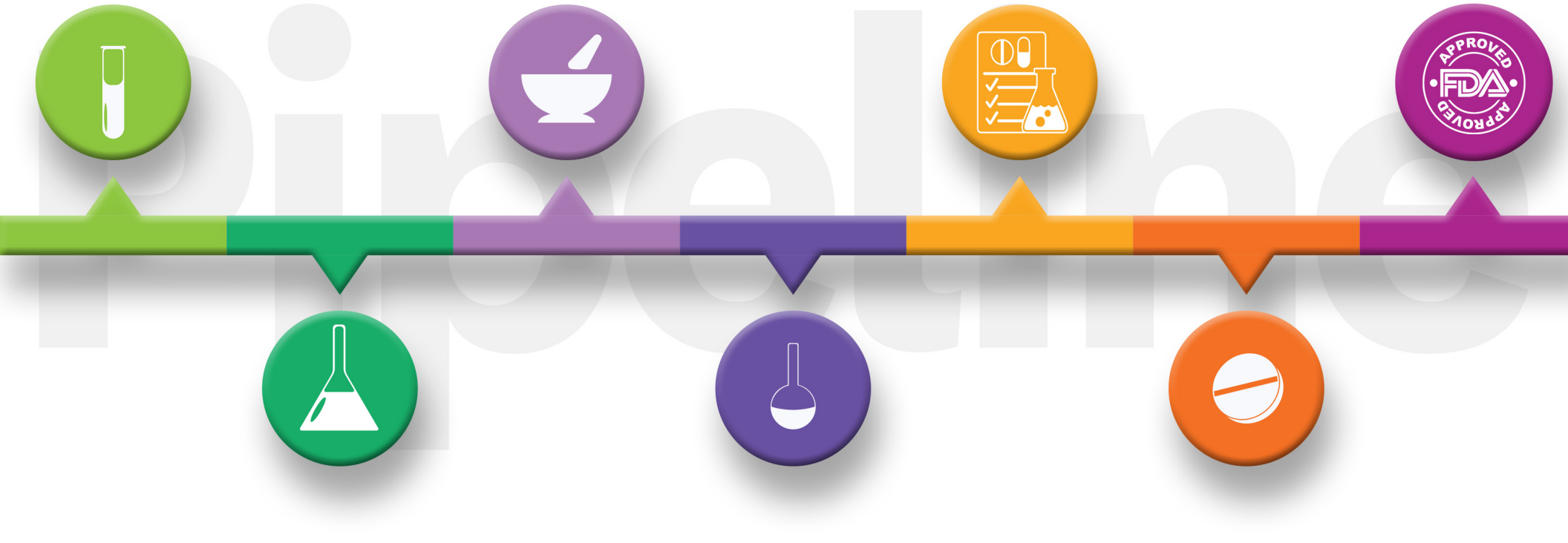

PIPELINE REPORT: JULY 2024

AcariaHealth™
Specialty Pharmacy

This quarterly publication is developed by our Clinical Pharmacy Drug Information team to provide additional drug pipeline information and insights to help health care leaders prepare for shifts in prescription drug management.

TABLE OF CONTENTS

SPECIALTY

PIPELINE REPORT: **JULY 2024**

Highlights

APPROVED: ELEVIDYS *(delandistrogene moxeparvovec-rokl)* for the treatment of Duchenne muscular dystrophy (DMD), now for ambulatory and non-ambulatory patients aged 4 years and up.

APPROVED: OHTUVAYRE *(ensifentrine)* for the treatment of chronic obstructive pulmonary disease (COPD) representing the first new mechanism of action to be approved for use in COPD in over 20 years.

APPROVED: KISUNLA *(donanemab-azbt)* for the treatment of early Alzheimer's disease.

 | Data is current as of 7/25/2024

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
GASTROENTEROLOGY					
IQIRVO® *elafibranor* oral tablet Available through **AcariaHealth**	Ipsen/ Genfit	Primary biliary cholangitis (PBC)	6/10/2024	• Accelerated FDA approval for the treatment of PBC in combination with ursodeoxycholic acid (UDCA) in adults who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA. This indication was approved under accelerated approval based on reduction of alkaline phosphatase. Improvement in survival or prevention of liver decompensation events have not been demonstrated. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s). • The IQIRVO Prescribing Infromation includes the following Limitation of Use: Use of IQIRVO is not recommended in patients who have or develop decompensated cirrhosis (e.g., ascites, variceal bleeding, hepatic encephalopathy). • Will compete with OCALIVA® for the same indication. • Projected impact: cost replacement of existing therapy.	$139,430/year
HEMATOLOGY					
VOYDEYA™ *danicopan* oral tablets	AstraZeneca	Paroxysmal nocturnal hemoglobinuria (PNH)	4/9/2024	• FDA-approved for use as add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis (EVH) in adults with PNH. • Prescribing Information includes a Boxed Warning re: an increased risk of serious and life-threatening infections caused by encapsulated bacteria. • VOYDEYA™ is available only through a restricted program called the VOYDEYA™ Risk Evaluation and Mitigation Strategy (REMS). • Projected impact: incremental cost increase.	$67,014/year

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
BEQVEZ™ *fidanacogene elaparvovec-dzkt* IV infusion	Pfizer	Hemophilia B	4/25/2024	• FDA-approved for the treatment of adults with moderate to severe hemophilia B (congenital factor IX deficiency) who currently use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes, and do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test. • Current standard of care is factor IX (FIX) replacement therapy. • Will compete with HEMGENIX® gene therapy for the same indication. • Projected impact: cost replacement of existing therapies.	$3.5 million/ one-time treatment
PIASKY® *crovalimab-akkz* IV infusion followed by SC injections	Genentech	Paroxysmal nocturnal hemoglobinuria (PNH)	6/20/2024	• FDA-approved for the treatment of adult and pediatric patients ≥ 13 years of age with PNH and body weight of ≥ 40 kg. • Will compete with BKEMV™ (once launched), EMPAVELI® (SC injection), EPYSQLI® (once launched), SOLIRIS®, and ULTOMIRIS® as another injectable complement-mediated therapy. • The PIASKY® Prescribing Information includes a Boxed Warning re: serious meningococcal infections. Competitor therapies have the same or similar Boxed Warning on their labels. • Projected impact: cost replacement of existing therapies.	Year 1: $353,800 Year 2 and thereafter: $212,280/year
IMMUNOLOGY					
XOLREMDI™ *mavorixafor* oral capsule	X4 Pharmaceuticals	Warts, hypogammaglobulinemia, infections, and myelokathexis (WHIM) syndrome	4/26/2024	• FDA-approved for use in patients > 12 years of age with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. • The prevalence of WHIM syndrome in the general population has been estimated at 0.2 per million live births. • Projected impact: incremental cost increase in a small population.	Weight-based dosing: $372,300-$496,400/ year

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
MUSCULOSKELETAL CONDITIONS					
ELEVIDYS® *delandistrogene moxeparvovec-rokl* IV infusion Available through **AcariaHealth**	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	6/20/2024	• **New indication for an existing gene therapy.** • FDA-approved for the treatment of DMD in patients ≥ 4 years of age who have a confirmed mutation in the DMD gene and who are ambulatory (regular approval) or non-ambulatory (accelerated approval). The accelerated approval for non-ambulatory patients was based on expression of ELEVIDYS® microdystrophin levels. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). • ELEVIDYS® was previously granted accelerated approval for the treatment of patients with DMD who were 4 through 5 years of age and still ambulatory. That accelerated indication has been converted to regular approval and expanded to include a broader age range. • The expanded indications were approved despite ELEVIDYS® having failed to meet the primary endpoint of improvement over placebo on the North Star Ambulatory Assessment (NSAA) total score in two separate Phase 3 trials. Still, the FDA considered the totality of the evidence which included positive trends in secondary endpoint results from one of these two trials as well as microdystrophin data from an additional open-label study. • The FDA approval of the expanded indications for a larger age range and non-ambulatory patients is expected to greatly increase utilization of ELEVIDYS®. • Projected impact: significant cost increase.	$3.2 million/ one-time treatment

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
NEUROLOGY					

KISUNLA™
donanemab-azbt
IV infusion
	Eli Lilly & Co	Early Alzheimer's disease (AD)	7/2/2024	• Approved for the treatment of AD in patients with mild cognitive impairment or mild dementia stage of disease, the population in which treatment was initiated in the clinical trials.
				• The KISUNLA™ Prescribing Information includes a Boxed Warning regarding amyloid-related imaging abnormalities (ARIA) and their increased risk in patients with ApoE ε4 homozygosity. The LEQEMBI® Prescribing Information contains the same Boxed Warning.
				• Final results of the Phase III TRAILBLAZER-ALZ 2 trial demonstrated a slowing of clinical decline of 35% at 18 months in people who received KISUNLA™ compared to placebo.
				  • Results were stratified by baseline tau levels, either low-medium tau level or low-medium plus high tau levels. People who were the least advanced in the disease experienced the strongest results with KISUNLA™.
				  • Those individuals with low/medium tau levels showed a significant slowing of decline of 35% on the integrated Alzheimer's Disease Rating Scale (iADRS) while on KISUNLA™ compared with those who received placebo. In the combined population, the response to treatment was also statistically significant, at 22%.
				  • In this study, the incidence of serious ARIA with edema was 1.6% and of serious ARIA with hemosiderin deposition was 4.7%, including two participants whose death was attributed to ARIA and a third participant who died after an incident of serious ARIA.
				• Discontinuation of KISUNLA™ dosing may be considered based on reduction of amyloid plaques to minimal levels on amyloid PET imaging.
				• KISUNLA™ will compete with LEQEMBI®, and will likely be subject to the same coverage restrictions imposed by the CMS National Coverage Determination (NCD) for this class of agents.
				• Projected impact: cost replacement of existing therapies.
					6 months of treatment: $12,522
					12 months of treatment: $32,000
					18 month of treatment: $48,696

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ONCOLOGY					
ABECMA® *idecabtagene vicleucel* IV infusion	Bristol Myers Squibb	Relapsed or refractory multiple myeloma (RRMM)	4/4/2024	• **New indication for an existing CAR T-cell therapy.** · FDA-approved for the treatment of adult patients with RRMM after two or more prior lines of therapy including an immunomodulatory agent (IMiD), a proteasome inhibitor (PI), and an anti-CD38 monoclonal antibody. · Expands use of ABECMA® for third line of therapy or later; was previously FDA-approved for use as fifth line or later therapy. · A previously anticipated update was added to the Prescribing Information Boxed Warning re: the increased risk of secondary hematological malignancies. · Projected impact: cost replacement of existing therapies.	$498,408/ one-time treatment
CARVYKTI® *ciltacabtagene autoleucel* IV infusion	Janssen	RRMM	4/5/25	• **New indication for an existing CAR T-cell therapy.** · FDA-approved for the treatment of adult patients with RRMM, who have received at least one prior line of therapy, including a PI and an IMiD, and are refractory to lenalidomide. · Expands use of CARVYKTI® for second line of therapy or later; was previously FDA-approved for use as fifth line or later therapy. · A previously anticipated update was added to the Prescribing Information Boxed Warning re: the increased risk of secondary hematological malignancies. · Projected impact: cost replacement of existing therapies.	$522,056/ one-time treatment
ANKTIVA® *nogapendekin alfa inbakicept-pmln* intravesical instillation	ImmunityBio	Non-muscle invasive bladder cancer (NMIBC)	4/22/2024	· FDA-approved for use in combination with Bacillus Calmette-Guérin (BCG) for the treatment of adult patients with BCG-unresponsive NMIBC with carcinoma in situ (CIS) with or without papillary tumors. · The recommended duration of treatment is until disease persistence after second induction, disease recurrence or progression, unacceptable toxicity, or a maximum of 37 months. · Will compete with ADSTILADRIN® within the same patient population pool. · Projected impact: cost replacement of existing therapies.	Year 1: $537,000 Years 2 and 3: $214,800/year Year 4 (final dose): $107,400

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
OJEMDA™ *tovorafenib* oral tablets and oral suspension	Day One Biopharmaceuticals	Pediatric low-grade glioma (pLGG)	4/23/2024	• Accelerated FDA approval for the treatment of patients ≥ 6 months of age with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. This application was granted accelerated approval based on overall response rate and duration of response. Continued approval may be contingent upon verification of clinical benefit in confirmatory trials. • Though rare, pLGG is the most common brain tumor diagnosed in children, accounting for 30% – 50% of all central nervous system tumors. There are no approved therapies and no standard of care for relapsed or refractory disease. • Projected impact: cost increase in a small population.	Up to $661,362/year (dosing based on body surface area)
BREYANZI® *lisocabtagene maraleucel* IV infusion	Bristol Myers Squibb	Follicular lymphoma (FL) and mantle cell lymphoma (MCL)	5/15/2024 (FL) & 5/30/2024 (MCL)	• **New indications for an existing CAR T-cell therapy.** • FDA-approved for the treatment of adults with relapsed or refractory FL who have received ≥ 2 prior lines of systemic therapy and for the treatment of adults with relapsed or refractory MCL who have received ≥ 2 prior lines of systemic therapy, including a Bruton tyrosine kinase (BTK) inhibitor. • Would compete with KYMRIAH® and YESCARTA® for the FL indication and with TECARTUS® for the MCL indication. • Projected impact: cost replacement of existing therapies.	$487,477/ one-time treatment
IMDELLTRA™ *tarlatamab-dlle* IV infusion	Amgen	Small-cell lung cancer (SCLC)	5/16/2024	• Accelerated FDA approval for the treatment of adult patients with extensive stage SCLC with disease progression on or after platinum-based chemotherapy. The accelerated approval was based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial. • Prescribing Information includes a Boxed Warning re: cytokine release syndrome and neurologic toxicity including immune effector cell-associated neurotoxicity syndrome. • Projected impact: cost replacement of existing therapies.	Year 1: $376,500 Year 2 and thereafter: $390,000/ year

 | Data is current as of 7/25/2024

CONTINUED

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
RYTELO™ *imetelstat* IV infusion	Geron	Myelodysplastic syndromes (MDS)	6/6/2024	· FDA-approved for the treatment of adult patients with low- to intermediate-1 risk myelodysplastic syndromes (MDS) with transfusion-dependent anemia requiring ≥ 4 red blood cell units over 8 weeks who have not responded to or have lost response to or are ineligible for erythropoiesis-stimulating agents (ESA). · Will compete with REBLOZYL® within the same patient population pool. · Projected impact: cost replacement of existing therapies.	$357,000/year
KEYTRUDA® *pembrolizumab* IV infusion	Merck & Co.	Endometrial cancer	6/17/2024	• **New indication for an existing agent.** · FDA-approved for use in combination with carboplatin and paclitaxel, followed by KEYTRUDA® as a single agent, for the treatment of adult patients with primary advanced or recurrent endometrial carcinoma. · Current NCCN guidelines already support use for the proposed indication as preferred first-line therapy for recurrent disease. · Projected impact: cost replacement of existing therapies.	$204,072/year
RESPIRATORY					
OHTUVAYRE™ *ensifentrine* nebulized inhalation therapy Available through **AcariaHealth**	Verona Pharma	Chronic obstructive pulmonary disease (COPD)	6/26/2024	· FDA-approved for the maintenance treatment of COPD in adult patients. · Has a novel mechanism of action as a selective dual inhibitor of phosphodiesterase (PDE) 3 and PDE4 that combines bronchodilator and non-steroidal anti-inflammatory effects in product. · Will likely be used as an adjunct to existing standard of care therapies such as inhaled bronchodilators and corticosteroids. · Projected impact: incremental cost increase.	$35,400/year

 | Data is current as of 7/25/2024

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
CARDIOVASCULAR DISEASE						
AG10 *acoramidis* oral therapy	BridgeBio Pharmaceuticals/ Eidos Therapeutics	Cardiomyopathy (CM)	Transthyretin stabilizer	• Proposed for the treatment of transthyretin amyloidosis cardiomyopathy (ATTR-CM). • Would compete with VYNDAQEL® and VYNDAMAX® which are FDA-approved for the same indication.	$300,000/ year	11/29/2024
COAGULATION DISORDERS						
PF-06741086* *marstacimab* subcutaneous (SC) injection	Pfizer	Hemophilia A or B	Tissue factor pathway inhibitor (TFPI)-neutralizing antibody	• Proposed for the prevention of bleeding episodes in patients ≥ 12 years of age with hemophilia A or B without inhibitors. • Once weekly SC injection. • For hemophilia A, would compete directly with HEMLIBRA® and factor VIII (FVIII) replacement therapy, while providing a chronic therapy alternative to ROCTAVIAN™ gene therapy. • For hemophilia B, would compete directly with FIX replacement therapy while providing a chronic therapy alternative to HEMGENIX® gene therapies.	$500,000/ year	4Q 2024

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

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

SPK-8011*
dirloctogene samoparvovec
IV infusion
	Spark Therapeutics and Roche	Hemophilia A	Gene therapy	• Proposed for the treatment of adults with severe disease.
				• Current standard of care is FVIII replacement therapy or HEMLIBRA®.
				• In the ongoing Phase I/II trial, FVIII expression was durable and sustained within mild hemophilia A range for most participatnts, with up to 6.5 years of follow-up.
				  • Clinically meaningful reductions were observed in median annualized bleed rate (ABR) (88–99%) and annualized FVIII infusion rate (AIR) (97–98%) compared with historical baseline.
				  • There were no deaths, no thrombotic events and no FVIII inhibitor development reported.
				• The Phase III Keystone-1 trial has launched.
				• Would compete with ROCTAVIAN™ gene therapy for the same indication.
					$2-3 million/ one-time treatment	2025

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

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

SB-525*
giroctocogene fitelparvovec
IV infusion
	Pfizer and Sangamo Therapeutics	Hemophilia A	Gene therapy	• Proposed for the treatment of adults with severe disease.
				• Current standard of care is FVIII replacement therapy or HEMLIBRA®.
				• The Phase 3 AFFINE study achieved its primary objective of non-inferiority, as well as superiority, of total ABR from Week 12 through ≥ 15 months of follow up post-infusion with SB-525 compared with routine FVIII replacement therapy. SB-525 demonstrated a statistically significant reduction in mean total ABR compared to the pre-infusion period (1.24 vs 4.73; p = 0.0040).
				  • Key secondary endpoints were met and also demonstrated superiority compared to FVIII prophylaxis: 84% of participants maintained FVIII activity > 5% at 15 months post-infusion (p = 0.0086), and the mean treated ABR showed a 98.3% reduction from 4.08 in the pre-infusion period to 0.07 post-infusion with SB-525 (from Week 12 up to ≥ 15 months [15-44 months]; p < 0.0001). Among all dosed participants, one participant (1.3%) returned to FVIII prophylaxis post-SB-525 infusion.
				  • SB-525 was generally well tolerated. Transient elevated FVIII levels ≥ 150% were observed in 49.3% of dosed participants, with no impact on efficacy and safety results. Serious adverse events were reported in 15 patients (20%), including 13 events reported by 10 patients (13.3%) assessed as related to treatment.
				• Would compete with ROCTAVIAN™ gene therapy for the same indication.
					$2-3 million/ one-time treatment	2025

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
DERMATOLOGY						
CIM331 *nemolizumab* SC injection	Galderma	Atopic dermatitis (AD) and prurigo nodularis (PN)	Anti-IL-31RA monoclonal antibody	· Proposed for the treatment of adolescents and adults with moderate to severe AD and for the treatment of pruritus associated with PN. · Once monthly injection. · Would compete with DUPIXENT® and ADBRY® for the AD indication.	$45,000/year	3Q 2024
ENDOCRINOLOGY						
TRANSCON PTH *palopegteriparatide* SC injection	Ascendis Pharmaceuticals	Hypoparathyroidism	Prodrug of parathyroid hormone	· Proposed for the treatment of adult patients with hypoparathyroidism. · Would compete with NATPARA® until NATPARA® manufacturing ceases at the end of 2024.	$130,000/ year	8/14/2024
MIPLYFFA *arimoclomol* oral therapy	Zevra Therapeutics	Niemann-Pick type C (NPC) disease	Heat-shock protein modulator	· Proposed for the treatment of NPC disease. · NPC is an ultra-rare, progressive, neurodegenerative genetic disorder with a prevalence of approximately one person per million in the U.S. · Would potentially compete with IB1001, if FDA-approved.	$400,000/ year	9/21/2024
IB1001 *N-acetyl-L-leucine* granules for oral suspension	IntraBio Inc.	NPC disease	Neuroprotective agent	· Proposed for the treatment of NPC disease. · Would potentially compete with MIPLYFFA™ (arimoclomol), if FDA-approved.	$400,000/ year	9/24/2024
AT-007 *govorestat* oral therapy	Applied Therapeutics	Galactosemia	Aldose reductase inhibitor	· Proposed for the treatment of classic galactosemia. · There are approximately 3,000 patients with galactosemia in the U.S.	$400,000/ year	11/28/2024

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
IONIS-APOCIII-LRx* *olezarsen* subcutaneous injection	Ionis Pharmaceuticals	Familial chylomicronemia syndrome (FCS)	Anti-sense oligonucleotide	· FCS is a rare, genetic disease characterized by extremely elevated triglyceride levels, impacting ~1-2/1,000,000 people worldwide. · There are no FDA-approved therapies for the treatment of FCS. Patients currently rely solely on nutrition management through extremely restrictive diets.	$500,000/ year	12/19/2024
crinecerfont oral capsule and oral solution	Neurocrine Biosciences	Congenital adrenal hyperplasia (CAH)	Corticotropin-releasing factor type 1 receptor antagonist	· Proposed for the treatment of children, adolescents and adults with classic CAH. · CAH is a rare genetic condition that occurs in ~1/10,000 people. Approximately 95% of CAH cases are classic CAH and are caused by a deficiency of the 21-hydroxylase (21-OHD) enzyme. If left untreated, CAH can result in salt wasting, dehydration, and even death. · The current standard of care for CAH is the use of glucocorticoids at supraphysiologic doses, which can be associated with serious and significant complications of steroid excess, including metabolic issues such as weight gain and diabetes, cardiovascular disease, and osteoporosis.	$400,000/ year	12/29/2024 (oral capsule) 12/30/2024 (oral solution)
MTP133* *elamipretide* SC injection	Stealth BioTherapeutics	Barth syndrome	Mitochondrial cardiolipin stabilizer	· Barth syndrome is a rare metabolic disorder characterized by skeletal muscle weakness, delayed growth, fatigue, varying degrees of physical disability, cardiomyopathy, neutropenia and methylglutaconic aciduria. · The estimated incidence of Barth syndrome is between one in 300,000 to 400,000 births. · There are currently no FDA-approved therapies for Barth syndrome; treatment is focused on reducing symptoms and preventing complications.	$500,000/ year	1/29/2025

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
GASTROENTEROLOGY						
MBX-8025 *seladelpar* oral capsule	Gilead	PBC	Peroxisome proliferator-activator receptor agonist	• Proposed for the management of PBC, including pruritus in adults without cirrhosis or with compensated cirrhosis (Child Pugh A) who are inadequate responders or intolerant to UDCA. • Would compete with OCALIVA®, potentially with a lower rate of pruritus.	$110,000/ year	8/14/2024
ZP1848* *glepaglutide* SC injection	Zealand Pharma	Short bowel syndrome (SBS)	GLP-2 analog	• Proposed for the treatment of SBS dependent on parenteral support. • Twice weekly injections with an auto-injector pen that will compete with GATTEX for the same indication.	$500,000/ year	12/22/2024
HEMATOLOGY						
Kresladi* *marnetegragene autotemcel* IV infusion	Rocket Pharmaceuticals	Leukocyte adhesion deficiency-I (LAD-I)	Lentiviral vector-based gene therapy	• LAD-I is a rare genetic condition that results in recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. • LAD-I is estimated to occur in ~1:1,000,000 people worldwide. • Bone marrow transplant is the only available curative therapy, but mortality in patients with severe LAD-I remains at 60-75% prior to 2 years of age and survival beyond 5 years of age is uncommon. • On 6/28/2024 the FDA issued a Complete Response Letter, citing issues related to manufacturing of the product. Rocket expects to quickly address the issues outlined in the Letter and resubmit the BLA.	$3-4 million/ one-time treatment	TBD

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
IMMUNOLOGY						
CTP-543 *deuruxolitinib* oral therapy	Sun Pharmaceuticals	Alopecia areata (AA)	JAK1 and JAK2 inhibitor	• Proposed for the treatment of moderate-to-severe AA. • Would compete with OLUMIANT® and LITFULO™, two other JAK inhibitors that are FDA-approved for AA.	$45,000/year	8/6/2024
SNDX-6352 *axatilimab* IV infusion	Incyte and Syndax	Chronic graft vs. host disease (cGvHD)	Anti-colony stimulating factor-1 receptor (CSF-1R) monoclonal antibody	• Proposed for the treatment of cGVHD after failure of at least two prior lines of systemic therapy. • cGVHD is estimated to develop in approximately 40% of allogeneic hematopoietic stem cell transplantation (HSCT) recipients.	$200,000/ year	8/28/2024
CSL312* *garadacimab* SC injection	CSL Behring	Hereditary angioedema (HAE)	Factor XIIa-inhibitory monoclonal antibody	• Proposed for the prevention of hereditary angioedema attacks in patients ≥ 12 years of age. • Once monthly SC injection. • Would compete with other HAE prophylactic therapies including CINRYZE®, HAEGARDA®, ORLADEYO®, and TAKHZYRO®.	$500,000/ year	10/14/2024
Ryoncil *remestemcel-L* IV injection	Mesoblast	Acute graft vs. host disease (aGVHD)	Stem cell therapy	• Proposed for treatment of children with steroid refractory aGVHD (SR-aGVHD). • If FDA-approved, Ryoncil will be the first allogeneic off-the-shelf cellular medicine to be approved in the U.S. for children < 12 years of age with SR-aGVHD; Jakafi is FDA-approved for the treatment of SR-aGVHD in children > 12 years of age.	$350,000/4-week course of therapy	1/7/2025
MUSCULOSKELETAL CONDITIONS						
SI-6603 condoliase intraspinal injection	Ferring Pharmaceuticals	Lumbar disc herniation (LDH)	Reduces intervertebral disc pressure	• Proposed for the treatment of radicular leg pain associated with LDH. Current treatments include conservative treatments for temporal pain relief (rest and non-steroidal anti-inflammatory drugs [NSAIDs]) and surgical treatments.	$10,000/ one-time treatment	3/14/2025

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
PF-06939926* *fordadistrogene movaparvovec* IV infusion	Pfizer	DMD	Gene therapy	• One-time treatment. • The phase 3 CIFFREO trial did not meet its primary endpoint of improvement in motor function among boys 4 to 7 years of age compared to placebo. The primary endpoint in the final analysis was change in the NSAA at one year after gene therapy treatment. Key secondary endpoints, including 10-meter run/walk velocity and time to rise from floor velocity, also did not show a significant difference between the gene tx and placebo. • Prospects for an FDA approval now appear slim. If approved, however, this agent would compete with ELEVIDYS™ gene therapy.	$2-3 million/ one-time treatment	2025

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
NEUROLOGY						

UPSTAZA* *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 one 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.
					$3-4 million/ one-time treatment	11/13/2024

Expected to cost ≥ $500,000 per member.

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ONCOLOGY						
ADP-A2M4* *afamitresgene autoleucel* IV infusion	Adaptimmune	Soft tissue sarcoma	Melanoma-associated antigen 4 (MAGE A4) T-cell therapy	• Proposed for the treatment of advanced synovial sarcoma or myxoid/round cell liposarcoma (MRCLS). • Synovial sarcoma accounts for ~6% to 10% of all soft tissue sarcomas; MRCLS is one of the most common types of liposarcoma and makes up about 30% of all liposarcoma cases and 10% of all soft tissue sarcomas.	$500,000/ one-time treatment	8/4/2024
LYMPHIR *denileukin diftitox* IV infusion	Citius	Cutaneous T-cell lymphoma (CTCL)	Cytocidal agent	• Proposed for treatment of patients with relapsed or refractory CTCL after at least one prior systemic therapy. • I/Ontak is an enhanced formulation of previously FDA-approved Ontak, which was marketed in the U.S. from 1999 to 2014, when it was voluntarily withdrawn from the market. • I/Ontak maintains the same amino acid sequence but features improved purity and bioactivity over Ontak.	$300,000/ year	8/13/2024
AG881 *vorasidenib* oral therapy	Servier	Glioma	IDH1 and IDH2 inhibitor	• Proposed for the treatment of IDH-mutant diffuse glioma.	$300,000/ year	8/20/2024
REGN5458 *linvoseltamab* IV infusion	Regeneron	RRMM	BCMAxCD3 bispecific antibody	• Proposed for the treatment of adult patients with RRMM who have progressed after at least three prior therapies.	$350,000/ year	8/22/2024
KEYTRUDA *pembrolizumab* IV infusion	Merck & Co.	Mesothelioma	Programmed death receptor-1 (PD-1) inhibitor	• **New indication for an existing agent.** • Proposed for use in combination with chemotherapy for the first-line treatment of patients with unresectable advanced or metastatic malignant pleural mesothelioma.	$204,000/ year	9/25/2024

Expected to cost ≥ $500,000 per member.

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
revumenib oral therapy	Syndax Pharmaceuticals	Acute leukemia	Menin inhibitor	• Proposed for the treatment of adult and pediatric patients with relapsed or refractory KMT2A-rearranged acute leukemia.	$300,000/ year	9/26/2024
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	NSCLC	PD-1 inhibitor	• **New indication for an existing agent.** • Proposed for neoadjuvant therapy in combination with chemotherapy followed by surgery and adjuvant therapy, for the perioperative treatment of resectable Stage IIA to IIIB NSCLC. • Previously approved in the neoadjuvant setting; label expansion to include usage in the adjuvant setting.	$190,793/ year	10/8/2024
treosulfan intravenous infusion	Medexus Pharmaceuticals	Allogeneic hematopoietic stem cell transplantation (allo-HSCT)	Alkylating agent	• Proposed for use in combination with fludarabine as a preparative regimen for allo-HSCT in adult and pediatric patients. • Would compete with busulfan for the same indication.	$7,500/ course of therapy	10/30/2024

MCLA-128
zenocutuzumab
intravenous infusion
	Merus	NSCLC; pancreatic cancer	Bispecific IgG1 antibody	• Proposed for the following two indications:
				  • For the treatment of patients with advanced unresectable or metastatic NRG1 fusion (NRG1+) NSCLC, following progression with prior systemic therapy.
				  • For the treatment of patients with advanced unresectable or metastatic NRG1+ pancreatic cancer following progression with prior systemic therapy or who have no satisfactory alternative treatment options.
				• NRG1 fusions are rare, occurring in < 1% of solid tumors.
					$450,000/ year	11/6/2024

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

 | Data is current as of 7/25/2024

CONTINUED

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
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, in combination with chemotherapy.	$250,000/ year	11/9/2024
AUTO1* *obecabtagene autoleucel* IV infusion	Autolus Therapeutics	Acute lymphoblastic leukemia (ALL)	CAR T-cell therapy	· Proposed for the treatment of patients with relapsed/ refractory (r/r) adult B-cell ALL. · If approved, would have overlapping indications with TECARTUS® and KYMRIAH®. · May have an improved tolerability profile over existing alternatives. · Administered as two doses given 10 days apart.	$500,000/ one-time treatment	11/16/2024
inavolisib oral therapy	Genentech	Breast cancer	PI3K inhibitor	· Proposed for use in combination with palbociclib and fulvestrant for the treatment of PIK3CA-mutated, HR-positive, HER2-negative, locally advanced or metastatic breast cancer, following recurrence on or within 12 months of completing adjuvant endocrine treatment. · PIQRAY is FDA-approved for use in combo with fulvestrant for the same cancer type following progression on or after an endocrine-based regimen.	$300,000/ year	11/27/2024
ZW25 *zanidatamab* intravenous infusion	Zymeworks	Biliary tract cancer (BTC)	Anti-HER2 bispecific antibody	· Proposed for the treatment of patients with previously-treated, unresectable, locally advanced, or metastatic HER2-positive BTC.	$400,000/ year	11/29/2024

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

 | Data is current as of 7/25/2024

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
lazertinib oral therapy	Janssen	NSCLC	EGFR-targeting tyrosine kinase inhibitor	• Proposed for use in combination with RYBREVANT for the first-line treatment of adult patients with locally advanced or metastatic NSCLC with epidermal growth factor (EGFR) exon 19 deletions or exon 21 L858R substitution mutations. • Would compete with TAGRISSO-containing regimens for the same indication.	$200,000/ year	12/21/2024
TAB-CEL *tabelecleucel* IV infusion	Atara Biotherapeutics	Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)	Allogeneic, EBV-specific T-cell immunotherapy	• Proposed as monotherapy for treatment of adult and pediatric patients two years of age and older with EBV+ PTLD who have received at least one prior therapy. • EBV+ PTLD can impact patients who have undergone solid organ transplant (SOT) or an allogeneic HCT. Poor median survival of 0.7 months and 4.1 months for HCT and SOT, respectively, is reported in EBV+ PTLD patients for whom rituximab ± chemotherapy failed. • Patients received a median of 2 treatment cycles in the pivotal trial for Tab-cel.	$300,000/ 35-day treatment cycle	1/15/2025
DATO-DXd *datopotamab deruxtecan* IV infusion	AstraZeneca	NSCLC	TROP2-directed DXd antibody drug conjugate	• Proposed for the treatment of adult patients with locally advanced or metastatic NSCLC.	$350,000/ year	1/29/2025
OPHTHALMOLOGY						
NT-501* *revakinagene taroretcel* intraocular implant	Neurotech	Macular telangiectasia type 2 (MacTel)	Ciliary neurotrophic factor (CNTF) cell therapy	• MacTel is a neurodegenerative disease resulting in photoreceptor atrophy and loss of vision. • Treatment with NT-501 significantly reduced anatomical disease progression through 24 months in two phase 3 studies.	$500,000/ one-time treatment	12/17/2024

 | Data is current as of 7/25/2024

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
RESPIRATORY DISEASE						
vanzacaftor/tezacaftor/deutivacaftor oral therapy	Vertex	Cystic fibrosis (CF)	Cystic fibrosis transmembrane conductance regulator (CFTR) modulator	• Proposed for the treatment of CF in patients ≥ 6 years of age who have at least one F508del mutation or another responsive mutation in the CFTR gene. • Will compete with Trikafta within the same indication.	$350,000/ year	1/2/2025
SURGERY						
HUMACYL *human acellular vessel* implantable tissue	Humacyte	Vascular trauma	Decellularized bioengineered blood vessels	• Proposed for urgent arterial repair following extremity vascular trauma in adults when synthetic graft is not indicated, and when autologous vein use is not feasible. • Off-the-shelf, bioengineered tissue; infection-resistant, universally implantable conduit for use in vascular repair.	$15,000/unit	8/10/2024

 | Data is current as of 7/25/2024

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
DERMATOLOGY						
SELARSDI™ *ustekinumab-aekn* subcutaneous (SC) injection	Alvotech and Teva Pharmaceuticals	STELARA®	Plaque psoriasis, psoriatric arthritis	FDA approval: 4/16/2024	No	• Is the second STELARA® biosimilar, after WEZLANA™. • Selarsdi is expected to be marketed in the U.S. on or after February 21, 2025, following a settlement agreement with Janssen, the manufacturer of Stelara.
PYZCHIVA® *ustekinumab-ttwe* SC injection	Samsung Bioepsis and Sandoz	STELARA®	Plaque psoriasis, psoriatic arthritis, Crohn's disease, ulcerative colitis	FDA approval: 6/28/2024	No	• Is the third STELARA® biosimilar, after SELARSDI™ and WEZLANA™. • Pyzchiva is expected to be marketed in the U.S. in February 2025, in accordance with a settlement and license agreement with Janssen.
FYB202 *ustekinumab* SC injection	Formycon and Fresenius Kabi	STELARA®	Plaque psoriasis	BLA is under FDA review (BsUFA date: 9/30/2024)	No	• Would be a subsequent STELARA® biosimilar, after PYZCHIVA®, SELARSDI™, and WEZLANA™.
DMB-3115 *ustekinumab* SC injection	Accord BioPharma	STELARA®	Plaque psoriasis	BLA is under FDA review (BsUFA date: 4Q 2024)	No	• Would be a subsequent STELARA® biosimilar, after PYZCHIVA®, SELARSDI™, and WEZLANA™.

 | Data is current as of 7/25/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
ENDOCRINOLOGY						
FKS518 *denosumab* SC injection	Fresenius Kabi	PROLIA®, XGEVA®	Osteoporosis; increasing bone mass when receiving aromatase inhibitor therapy, skeletal-related complications of multiple myeloma and of bone metastases; giant cell tumor of the bone; hypercalcemia of malignancy	BLA is under FDA review (BsUFA date: 3/27/2025)	No	• Would be the third denosumab biosimilar, after JUBBONTI® and WYOST®.
HEMATOLOGY						
BKEMV™ *eculizumab-aeeb* IV infusion	Amgen	SOLIRIS®	Paroxysmal nocturnal hemoglobinuria, atypical uremic hemolytic syndrome	FDA approval: 5/28/2024	No	• Is the first approved SOLIRIS® biosimilar product. • Anticipated U.S. launch is in March 2025 due to a patent litigation agreement with Alexion (Soliris manufacturer).

 | Data is current as of 7/25/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
NYPOZI™ *filgrastim-txid* SC or IV injection	Tanvex BioPharma	NEUPOGEN®	Febrile neutropenia, to reduce the time to neutrophil recovery and the duration of fever, in patients with acute myeloid leukemia, to reduce the duration of neutropenia and neutropenia-related clinical sequelae in patients with non-myeloid malignancies, hematopoietic progenitor cells mobilization, congenital neutropenia, cyclic neutropenia, or idiopathic neutropenia, hematopoietic syndrome of acute radiation syndrome	FDA approval: 6/28/2024	Yes	• Is the fourth NEUPOGEN biosimilar after NIVESTYM®, RELEUKO®, and ZARXIO®.
EPYSQLI® *eculizumab-aagh* IV infusion	Samsung Bioepis	SOLIRIS®	Paroxysmal nocturnal hemoglobinuria, atypical uremic hemolytic syndrome	FDA approval: 7/19/2024	No	• Is the second approved SOLIRIS® biosimilar product after BKEMV™. • Anticipated U.S. launch and pricing information have not been publicly disclosed.

 | Data is current as of 7/25/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
IMMUNOLOGY						
HYRIMOZ® *adalimumab-adaz* SC injection	Sandoz	HUMIRA®	RA; JIA; psoriatic arthritis; ankylosing spondylitis; Crohn's disease; ulcerative colitis; plaque psoriasis; hidradenitis suppurativa (HS); uveitis	FDA approval: 4/5/2024 (interchangeability status)	Yes	• Is the first interchangeable biosimilar version of the 80 mg/0.8 mL, 20 mg/0.2 mL and 10 mg/0.1 mL high-concentration products. • Is the second interchangeable biosimilar version of the 40 mg/0.4 mL high-concentration product, after SIMLANDI®. • Is the third interchangeable biosimilar product for the 40 mg/0.8 mL, 20 mg/0.4 mL and 10 mg/0.2 mL strengths, after ABRILADA™ and CYLTEZO®.
HADLIMA™ *adalimumab-bwwd* SC injection	Samsung Bioepsis	HUMIRA®	RA; JIA; psoriatic arthritis; ankylosing spondylitis; Crohn's disease; ulcerative colitis; plaque psoriasis; HS; uveitis	FDA approval of interchangeable status:6/28/2024	Yes	• ABRILADA™, CYLTEZO®, HYRIMOZ®, and SIMLANDI® also have interchangeable status.
CT-P39 *omalizumab* Celltrion	Celltrion	XOLAIR®	Asthma, chronic rhinosinusitis with nasal polyps, IgE-mediated food allergy, and chronic spontaneous urticaria	BLA is under FDA review (BsUFA date: 3/10/2025)	No	• Would be the first FDA-approved biosimilar to XOLAIR®.

 | Data is current as of 7/25/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
ONCOLOGY						
HERCESSI™ *trastuzumab-strf* IV infusion	Henlius Biotech and Accord BioPharma	HERCEPTIN®	Breast cancer; gastric or gastroesophageal junction (GEJ) cancer	FDA approval: 4/25/2024	Yes	· Is the sixth HERCEPTIN® biosimilar to be FDA-approved after HERZUMA®, KANJINTI®, OGIVRI®, ONTRUZANT® and TRAZIMERA®.
DRL_RI *rituximab* IV infusion	Dr. Reddy's Laboratories	RITUXAN®	Non-Hodgkin's lymphoma, RA	BLA is under FDA review (BsUFA date: 3Q 2024)	Yes	· Would be the fourth RITUXAN® biosimilar to be FDA-approved after RIABNI™, RUXIENCE®, and TRUXIMA®.
OPHTHALMOLOGY						
OPUVIZ™ *aflibercept-yszy* intraocular injection	Samsung Bioepis and Biogen	EYLEA®	Wet age-related macular degeneration (AMD), diabetic macular edema (DME), diabetic retinopathy (DR), macular edema following retinal vein occlusion (RVO)	FDA approval: 5/20/2024	No	· Is one of the first two FDA-approved biosimilars to EYLEA®, along with YESAFILI™. · FDA-approved with interchangeable status.
YESAFILI™ *aflibercept-jbvf* intraocular injection	Biocon	EYLEA®	Wet AMD, DME, DR, macular edema following RVO	FDA approval: 5/20/2024	No	· Is one of the first two FDA-approved biosimilars to EYLEA®, along with OPUVIZ™. · FDA-approved with interchangeable status.
AHZANTIVE® *aflibercept-mrbb* intraocular injection	Formycon	EYLEA®	Wet AMD, DME, DR, macular edema following RVO	FDA approval: 6/28/2024	No	· Is the third FDA-approved biosimilar to EYLEA®, after OPUVIZ™ and YESAFILI™.

 | Data is current as of 7/25/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
CTP42 *aflibercept* intraocular injection	Celltrion	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 3Q 2024)	No	• Would be a subsequent EYLEA® biosimilar, after AHZANTIVE®, OPUVIZ™, and YESAFILI™ .
ABP 938 *aflibercept* intraocular injection	Amgen	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 3Q 2024)	No	• Would be a subsequent EYLEA® biosimilar, after AHZANTIVE®, OPUVIZ™, and YESAFILI™ .

 | Data is current as of 7/25/2024

Generic Specialty Agents

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
eribulin mesylate	HALAVEN®	Gland Pharmaceuticals	5/3/2024
lanreotide acetate	SOMATULINE® DEPOT	Cipla	5/23/2024
deflazacort oral suspension	EMFLAZA®	Tris Pharmaceuticals	5/29/2024
edaravone (intravenous)	RADICAVA®	Gland Pharmaceuticals	6/11/2024
l-glutamine	ENDARI®	Novitium	7/15/2024
Pipeline Agents*			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**ANTICIPATED LAUNCH DATE**
nilotinib hydrochloride	TASIGNA®	Apotex	3Q 2024
dasatinib	SPRYCEL® (20, 50, 70, 80, 100, 140 mg)	Apotex	9/1/2024
octreotide acetate	SANDOSTATIN LAR®	Teva	2024
bendamustine oral solution	TREANDA®	Fresenius Kabi	2024
tolvaptan	JYNARQUE®	Lupin	4/23/2025
degarelix acetate	FIRMAGON®	Ferring Pharmaceuticals	1H2024
glycerol phenylbutyrate	RAVICTI®	Par/Endo	7/1/2025

***Includes generic agents with > 50% launch probability**

 | Data is current as of 7/25/2024

Glossary

Term	Definition
AA	alopecia areata
AADC	aromatic L-amino acid decarboxylase
ABR	annualized bleeding rate
AChR	acetylcholine receptor
AD	Alzheimer's disease
ALL	acute lymphoblastic leukemia
AIR	annualized infusion rate
AMD	age-related macular degeneration
AML	acute myeloid leukemia
ARIA	amyloid-related imaging abnormalities
ARSA	arylsulfatase A
AS	ankylosing spondylitis
ATTR-CM	transthyretin amyloidosis cardiomyopathy
ATTRv-PN	transthyretin-mediated amyloid polyneuropathy
BCG	bacillus Calmette-Guérin
BiTE	bispecific T-cell engager
BLA	biologics license application
BMI	body mass index
BRCA	breast cancer gene
BsUFA	Biosimilar User Fee Act

Term	Definition
BTC	biliary tract cancer
BTK	Bruton tyrosine kinase
CAH	congenital adrenal hyperplasia
CAR T-cell	chimeric antigen receptor T-cell
CD	Crohn's disease
CDC	Centers for Disease Control and Prevention
CDR-SB	Clinical Dementia Rating-Sum of Boxes
cGVHD	chronic graft vs. host disease
CIS	carcinoma in situ
CKD	chronic kidney disease
CLL	chronic lymphocytic leukemia
CMS	Centers for Medicare & Medicaid Services
COPD	chronic obstructive pulmonary disease
CRBSI	catheter-related bloodstream infection
cSCC	cutaneous squamous cell carcinoma
CTCL	cutaneous T-cell lymphoma
cTTP	congenital thrombotic thrombocytopenic purpura
CV	cardiovascular
DEB	dystrophic epidermolysis bullosa
DED	dry eye disease

Term	Definition
DLBCL	diffuse large B-cell lymphoma
DMD	Duchenne muscular dystrophy
DME	diabetic macular edema
DR	diabetic retinopathy
EB	epidermolysis bullos
EGFR	epidermal growth factor receptor
ERT	enzyme replacement therapy
ESA	erythropoiesis-stimulating agent
EVH	extravascular hemolysis
ET	essential thrombocythemia
FIX	factor IX
FVIII	factor VIII
FDA	Food and Drug Administration
FIGO	Federation Internationale de Gynecolgie et d'Obstetrique (in French); International Federation of Gynecology and Obstetrics (in English)
FL	follicular lymphoma
FOP	fibrodysplasia ossificans progressiva
GA	geographic atrophy
GCA	giant cell arteritis
GEJ	gastroesophageal junction

 | Data is current as of 7/25/2024

CONTINUED

Glossary

Term	Definition
GIP	glucose-dependent insulinotropic polypeptide
GLP-1	glucagon-like peptide-1
gMG	generalized myasthenia gravis
HAE	hereditary angioedema
HDAC	histone deacetylase
HER	human epidermal growth factor receptor
HF	heart failure
HR	hormone receptor
HS	hidradenitis suppurativa
HSCT	hematopoietic stem cell transplantation
ICER	Institute for Clinical and Economic Review
IMiD	immunomodulatory agent
IV	intravenous
JAK1	Janus Kinase 1
JAK2	Janus Kinase 2
JEB	junctional epidermolysis bullosa
JIA	juvenile idiopathic arthritis
LAD-I	leukocyte adhesion deficiency-I
LBCL	large B-cell lymphoma
LDH	lumbar disc herniation

Term	Definition
LRTD	lower respiratory tract disease
MACE	major adverse cardiovascular events
MCL	mantle cell lymphoma
MASH	metabolic dysfunction-associated steatohepatitis
mCRPC	metastatic castration-resistant prostate cancer
MDD	major depressive disorder
MDS	myelodysplastic syndrome
MI	myocardial infarction
MF	myelofibrosis
MLD	metachromatic leukodystrophy
MS	multiple sclerosis
MuSK	muscle-specific tyrosine kinase
NCD	National Coverage Determination
NMIBC	non-muscle invasive bladder cancer
NPC	Niemann-Pick type C
NSAA	North Star Ambulatory Assessment
NSCLC	non-small cell lung cancer
NTF	neurotrophic factor
PAH	pulmonary arterial hypertension

Term	Definition
PBC	primary biliary cholangitis
PD-L1	programmed death-ligand 1
PH1	primary hyperoxaluria type 1
PI	proteasome inhibitor
pJIA	polyarticular juvenile idiopathic arthritis
pLGG	pediatric low-grade glioma
PPD	post-partum depression
PN	prurigo nodularis
PNH	paroxysmal nocturnal hemoglobinuria
PsA	psoriatic arthritis
PSVT	paroxysmal supraventricular tachycardia
PV	polycythemia vera
RA	rheumatoid arthritis
RDEB	recessive dystrophic epidermolysis bullosa
REMS	Risk Evaluation and Mitigation Strategy
RRMM	relapsed or refractory multiple myeloma
RSV	respiratory syncytial virus
RVO	retinal vein occlusion
SC	subcutaneous
SCD	sickle cell disease

 | Data is current as of 7/25/2024

CONTINUED

Glossary

Term	Definition
SCLC	small cell lung cancer
sJIA	systemic juvenile idiopathic arthritis
SLL	small lymphocytic lymphoma
T2DM	type 2 diabetes mellitus
TDT	transfusion-dependent β-thalassemia
TFPI	tissue factor pathway inhibitor
TGF	transforming growth factor
UC	ulcerative colitis
UDCA	ursodeoxycholic acid
UTI	urinary tract infection
VEGF	vascular endothelial growth factor
VOC	vaso-occlusive crisis
VOE	vaso-occlusive event
WAC	Wholesale Acquisition Cost
WHIM	warts, hypogammaglobulinemia, infections, and myelokathexis

 | Data is current as of 7/25/2024

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