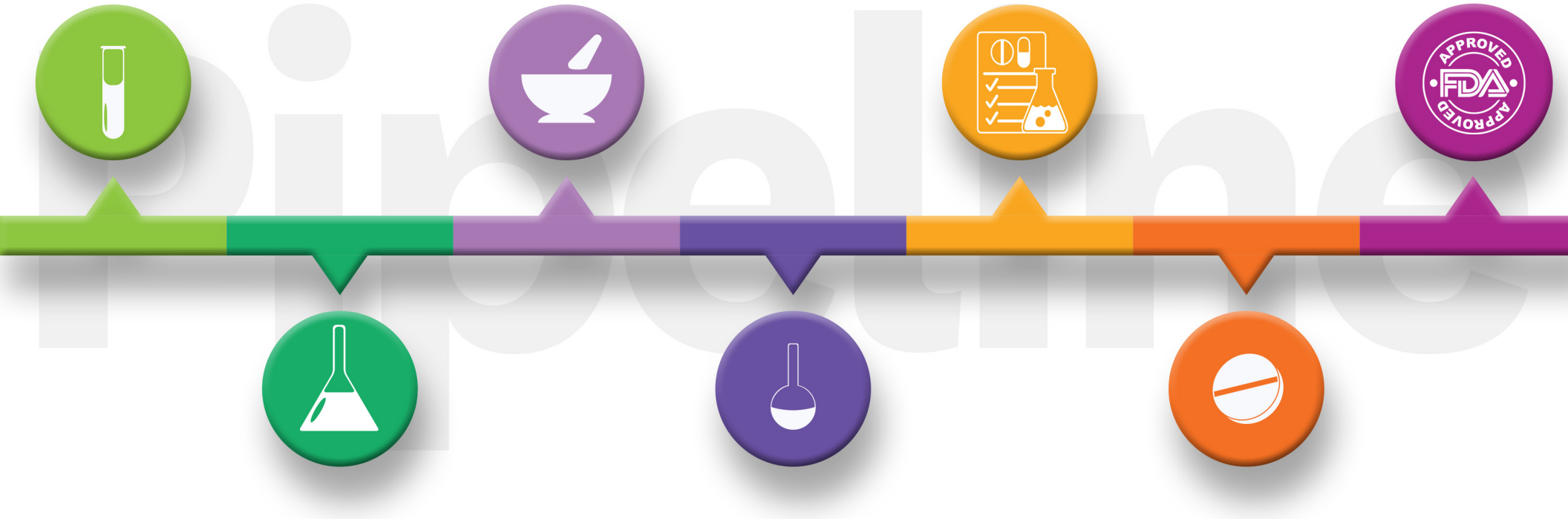

PIPELINE REPORT: APRIL 2024

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.

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SPECIALTY

PIPELINE REPORT: **APRIL 2024**

Highlights

APPROVED: WEGOVY® (semaglutide) is now approved to reduce the risk of major adverse cardiovascular events in those with established cardiovascular disease who are overweight or obese.

APPROVED: LENMELDY™ (atidarsagene autotemcel), a gene therapy for the treatment of early-onset metachromatic leukodystrophy, a condition thought to affect only one in every 100,000 births, with a high early mortality rate and taking over the top rank for most expensive therapy in the world.

APPROVED: REZDIFFRA™ (resmetirom) is the first agent approved for the treatment of metabolic dysfunction-associated steatohepatitis (MASH), formerly non-cirrhotic non-alcoholic steatohepatitis (NASH).

APPROVED: AMTAGVI™ (lifileucel), the first gene-modified cell therapy approved for the treatment of melanoma, a solid tumor, advancing the use of cell therapies beyond the historical realm of hematologic cancers.

 | Data is current as of 4/18/2024

Recent Specialty Drug Approvals

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

LENMELDY™ *atidarsagene autotemcel* intravenous (IV) infusion	Orchard Therapeutics	Metachromatic leukodystrophy (MLD)	3/18/2024	• FDA-approved for the treatment of children with pre-symptomatic late infantile, pre-symptomatic early juvenile or early symptomatic early juvenile MLD.
				• 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 of age from onset is estimated at 50% and 44% at 10 years of age for juvenile patients.
				• The Institute for Clinical and Economic Review (ICER) published a Final Evidence Report assessing the comparative clinical effectiveness and value of Lenmeldy for MLD.
				  • In the report, ICER assigned an Evidence Rating of high certainty of A (substantial net health benefit) vs. usual standard of care for Lenmeldy treatment in children with presymptomatic late-infantile and early juvenile forms of MLD.
				  • An Evidence Rating of moderate certainty of a B+ (moderate certainty of a small or substantial net health benefit with high certainty of at least a small net health benefit) vs. usual standard of care was assigned for early symptomatic early juvenile MLD.
				• Projected impact: cost increase in a small population.
					$4.25 million/ one-time treatment

GASTROENTEROLOGY					
REZDIFFRA™ *resmetirom* oral tablets	Madrigal Pharmaceuticals	Metabolic dysfunction-associated steatohepatitis (MASH) Formally non-alcoholic steatohepatitis (NASH)	3/14/2024	• The FDA granted accelerated approval for use in conjunction with diet and exercise for the treatment of adults with non-cirrhotic MASH with moderate to advanced liver fibrosis (consistent with Stages F2 to F3 fibrosis). • Is the first FDA-approved therapy for MASH, which is estimated to affect up to 6% of adults in the U.S. • Projected impact: cost increase due to the current high unmet need and the potentially large utilizing population.	$48,058/year

 | Data is current as of 4/18/2024

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
HEMATOLOGY					
CASGEVY™ *exagamglogene autotemcel* IV infusion	CRISPR Therapeutics and Vertex	Transfusion-dependent β-thalassemia (TDT)	1/16/2024	• FDA-approved for the treatment of TDT in patients ≥ 12 years of age. • Is the first FDA-approved gene therapy for TDT that utilizes CRISPR/Cas9 gene editing technology. • This gene therapy administration requires a myeloablative pre-conditioning regimen plus an extended post-treatment inpatient hospitalization. • Will compete with ZYNTEGLO™ gene therapy for the TDT indication. • Projected impact: cost replacement of existing therapy.	$2.2 million/ one-time treatment
AURLUMYN™ *iloprost* IV infusion	Eicos Sciences	Frostbite	2/13/2024	• FDA-approved for the treatment of severe frostbite in adults to reduce the risk of digit amputations. • Effectiveness was established in young, healthy adults who suffered frostbite at high altitudes. • Projected impact: incremental hospital cost increase in a small population.	Pending launch
VOYDEYA™ *danicopan* oral tablets	AstraZeneca	Paroxysmal nocturnal hemoglobinuria (PNH)	3/29/2024	• 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.	Pending launch
MUSCULOSKELETAL CONDITIONS					
DUVYZAT™ *givinostat* oral suspension	Italfarmaco	Duchenne muscular dystrophy (DMD)	3/21/2024	• FDA-approved for for the treatment of DMD in patients ≥ 6 years of age. • 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 regardless of gene mutation status. • Projected impact: incremental cost increase.	Pending launch

 | Data is current as of 4/18/2024

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ONCOLOGY					
KEYTRUDA® *pembrolizumab* IV infusion	Merck & Co	Gastric or gastroesophageal junction (GEJ) adenocarcinoma	1/12/2024	• **New indication for an existing agent.** • 1/12/2024: Approved for use in combination with chemoradiotherapy for the treatment of patients with FIGO 2014 Stage III-IVA cervical cancer. • Allows for use in earlier lines of cervical cancer therapy. • Projected impact: cost replacement of existing therapies.	$200,071/year
AMTAGVI™ *lifileucel* IV infusion	Iovance Biotherapeutics	Melanoma	2/16/2024	• The FDA granted accelerated approval for the treatment of adult patients with unresectable or metastatic melanoma previously treated with a PD-1 blocking antibody, and if BRAF V600 mutation positive, a BRAF inhibitor with or without a MEK inhibitor. • Examples of PD-1 blocking antibodies include KEYTRUDA®, OPDIVO®, OPDUALAG™, YERVOY®; examples of BRAF and MEK inhibitors include BRAFTOVI®, COTELLIC®, MEKINIST®, MEKTOVI®, TAFINLAR®, ZELBORAF®. • Previously only chemotherapy was available as an option at this stage of the disease. • Projected impact: incremental cost increase.	$515,000/ one-time treatment
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	Urothelial carcinoma	3/6/2024	• **New indication for an existing agent.** • FDA-approved for adult patients with unresectable or metastatic urothelial carcinoma, as first-line treatment in combination with cisplatin and gemcitabine. • Was previously FDA-approved for use with disease progression on or after platinum-containing chemotherapy, now approved for first-line use. • Projected impact: cost replacement of existing therapies.	$190,793/year
TEVIMBRA® *tislelizumab-jsgr* IV infusion	BeiGene	Esophageal squamous cell carcinoma (ESCC)	3/13/2024	• FDA-approved for use as a single agent for the treatment of adults with unresectable or metastatic ESCC after prior systemic chemotherapy that did not include a PD-(L)1 inhibitor. • Was evaluated for efficacy against chemotherapy as second-line therapy for advanced or metastatic ESCC. • Projected impact: cost replacement of existing therapies.	Pending launch

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
BREYANZI® *lisocabtagene maraleucel* IV infusion	Bristol Myers Squibb	Chronic lymphocytic leukemia (CLL); small lymphocytic lymphoma (SLL)	3/14/2024	• **New indication for an existing CAR T-cell therapy**. • FDA-approved for the treatment of patients with relapsed and/or refractory chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL) who received a prior Bruton tyrosine kinase inhibitor (BTKi) and B-cell lymphoma 2 inhibitor (BCL2i). • Is the first CAR T-cell therapy to be FDA-approved for these indications. • Projected impact: cost replacement of existing therapies.	$447,227/ one-time treatment
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.** • 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	4/5/2025	• **New indication for an existing CAR T-cell therapy.** • Approved for the treatment of adult patients with RRMM, who have received at least one prior line of therapy, including a proteasome inhibitor and an immunomodulatory agent, 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.	$478,950/ one-time treatment

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
RESPIRATORY DISEASE					
WINREVAIR™ *sotatercept-csrk* subcutaneous (SC) injection	Merck & Co.	Pulmonary arterial hypertension (PAH)	3/26/2024	• FDA-approved for the treatment of adults with PAH, World Health Organization (WHO) Group 1, to increase exercise capacity, improve WHO functional class (FC) and reduce the risk of clinical worsening events. • Self-administered SC injection every 21 days. • First potentially disease-modifying therapy in the PAH space, for use as an adjunct to stable background therapy. • Projected impact: incremental cost increase.	$238,000/year

 | Data is current as of 4/18/2024

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 VYNDAMEX® which are FDA-approved for the same indication.	$250,000/ year	11/29/2024
COAGULATION DISORDERS						

PF-06838435/SPK-9001*
fidanacogene elaparvovec
intravenous (IV) infusion
	Pfizer and Spark Therapeutics	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 annualized bleeding rate (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® gene therapy for the same indication.
				· The FDA accepted the BLA for review.
					$3-3.5 million/ one-time treatment	4/27/2024

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

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
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 therapy.	$450,000/ year	4Q 2024

SPK-8011*
dirloctogene samoparvovec
IV infusion
	Spark Therapeutics and 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 FVIII replacement therapy or HEMLIBRA®.
				· In the ongoing Phase I/II trial, FVIII 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 year.
				· 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 4/18/2024

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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	• For the treatment of adults with severe disease (~60% of the total hemophilia A population). • Current standard of care is FVIII 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 FVIII 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 1H 2024. • Meanwhile, updated results from the Phase I/II Alta trial showed that, among five patients receiving the highest dose of SB-525, mean FVIII 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						
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

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

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ENDOCRINOLOGY						
TransCon PTH *palopegteriparatide* SC injection	Ascendis Pharmaceuticals	Hypoparathyroidism	Prodrug of parathyroid hormone	· 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.	$130,000/ year	5/14/2024
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	8/28/2024
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
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.	$350,000/ year	2/8/2025

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
GASTROENTEROLOGY						
GFT505 *elafibranor* oral tablet	Genfit	Primary biliary cholangitis (PBC)	Peroxisome proliferator-activator receptor agonist	• Proposed for the treatment of PBC in adults with inadequate response to ursodeoxycholic acid. • Would compete with OCALIVA®, potentially with a lower rate of pruritus.	$110,000/ year	6/10/2024
MBX-8025 *seladelpar* oral capsule	CymaBay Therapeutics	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
HEMATOLOGY						
RG6107 *crovalimab* SC injection	Genentech	Paroxysmal nocturnal hemoglobinuria (PNH)	C5 complement inhibitor	• Proposed for the treatment of PNH in patients ≥ 12 years of age. • Self-administered SC injection once monthly. • Would compete with EMPAVELI® (SC injection), SOLIRIS® and ULTOMIRIS® as other injectable complement-mediated therapies.	$450,000/ year	6/15/2024
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 one in every one million 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.	$3-4 million/ one-time treatment	6/30/2024

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

 | Data is current as of 4/18/2024

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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	4/6/2024
X4P-001-RD *mavorixafor* oral capsule	X4 Pharmaceuticals	Warts, hypogammaglobulinemia, infections, and myelokathexis (WHIM) syndrome	CXCR antagonist	· Proposed for the treatment of patients > 12 years of age with WHIM syndrome. · The prevalence of WHIM syndrome in the general population has been estimated at 0.2 per million live births.	$200,000/ year	4/30/2024
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 subcutaneous injection. · Would compete with other HAE prophylactic therapies including CINRYZE®, HAEGARDA®, ORLADEYO®, and TAKHZYRO®.	$500,000/ year	10/14/2024

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

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
MUSCULOSKELETAL CONDITIONS						
ELEVIDYS®* *delandistrogene moxeparvovec-rokl* IV infusion	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	Gene therapy	· Proposed for the treatment of DMD patients with a confirmed mutation in the DMD gene. · The label expansion request seeks to remove age and ambulation restrictions from the currently approved indication, which includes only patients 4-5 years of age. · FDA approval of an expanded indication for a larger age range and/or expanding to non-ambulatory patients, could greatly increase utilization of ELEVIDYS®.	$3.2 million/ one-time treatment	6/21/2024
PF-06939926* *fordadistrogene movaparvovec* IV 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 III 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 4/18/2024

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Upcoming Specialty Products

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

LY3002813
donanemab
IV infusion
	Eli Lilly & Co	Early Alzheimer's Disease (AD)	Anti-amyloid monoclonal antibody	• Proposed for the treatment of early symptomatic AD.
				• Final results of the Phase III TRAILBLAZER-ALZ2 trial for donanemab for early Alzheimer's disease demonstrated a slowing of clinical decline of 35% at 18 months in people who received donanemab compared to placebo.
				  • 52% of participants with intermediate tau levels completed their course of treatment by one year and 72% completed by 18 months as a result of achieving plaque clearance.
				  • 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.
				• The FDA is planning an Advisory Committee meeting to discuss the safety and efficacy of donanemab, including the safety results in donanemab-treated patients and the efficacy implications of the unique trial design of the TRAILBLAZER-ALZ 2 study, including its limited-duration dosing regimen that allowed patients to complete treatment based on an assessment of amyloid plaque and the inclusion of participants based on tau levels. A meeting date has not yet been determined.
				• If approved, donanemab would compete with LEQEMBI®, and it would likely be subject to the same coverage restrictions imposed by the CMS National Coverage Determination (NCD) for this class of agents.
					$30,000/ year	2H 2024

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

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

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.
				• BLA submission was completed in 1Q 2024.
					$3-4 million/ one-time treatment	4Q 2024

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

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ONCOLOGY						
ANKTIVA® *nogapendekin alfa inbakicept* intravesical instillation	ImmunityBio	Non-muscle invasive bladder cancer (NMIBC)	Beta gamma T-cell receptor binder	• Proposed for use in combination with bacillus Calmette-Guérin (BCG), for the treatment of BCG-unresponsive non-muscle-invasive bladder cancer (NMIBC) carcinoma in situ with or without Ta or T1 disease.	$200,000/ year	4/23/2024
DAY101 *tovorafenib* oral therapy	Day One	Pediatric low-grade glioma (pLGG)	Pan-RF kinase inhibitor	• Proposed for the treatment of relapsed or progressive pLGG as a monotherapy.	$250,000/ year	4/30/2024
rivoceranib and camrelizumab oral therapy and IV infusion	Elevar Therapeutics	Hepatocellular carcinoma	Tyrosine kinase inhibitor + PD-1 inhibitor	• Proposed for combination use as a first-line treatment option for unresectable hepatocellular carcinoma.	$400,000/ year	5/16/2024
BREYANZI® *lisocabtagene maraleucel* IV infusion	Bristol Myers Squibb	Follicular lymphoma (FL) and mantle cell lymphoma (MCL)	CAR T-cell therapy	• **New indication for an existing CAR T-cell therapy.** • Proposed for the treatment of relapsed or refractory FL or for relapsed or refractory MCL after a Bruton tyrosine kinase inhibitor (BTKi). • Would compete with YESCARTA® for the FL indication and with TECARTUS® for the MCL indication.	$487,477/ one-time treatment	5/23/2024 (FL) 5/31/2024 (MCL)
AMG 757 *tarlatamab* IV infusion	Amgen	Small-cell lung cancer (SCLC)	DLL3-targeting bispecific T-cell engager (BiTE) therapy	• Proposed for the treatment of adults with advanced SCLC who have had disease progression on or after platinum-based chemotherapy.	$450,000/ year	6/12/2024
GRN163L *imetelstat* IV infusion	Geron Corporation	Myelodysplastic syndromes (MDS)	Telomerase inhibitor	• Proposed for the treatment of adult patients with transfusion-dependent anemia due to Low- or Intermediate-1 risk MDS who have failed to respond to, have lost response to, or are ineligible for, erythropoiesis-stimulating agents.	$200,000/ year	6/16/2024

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
KEYTRUDA *pembrolizumab* IV infusion	Merck & Co.	Endometrial cancer	Programmed death receptor-1 (PD-1) inhibitor	• **New indication for an existing agent.** • Proposed for use in combination with standard of care chemotherapy (carboplatin and paclitaxel), followed by KEYTRUDA® as a single agent for the treatment of 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.	$200,071/ year	6/21/2024
HER3-DXd *patritumab deruxtecan* IV infusion	Daiichi Sankyo and Merck & Co.	Non-small cell lung cancer (NSCLC)	HER3-directed antibody drug conjugate	• Proposed for the treatment of patients with metastatic or locally advanced EGFR-mutated NSCLC with disease progression on or after treatment with two or more systemic therapies.	$350,000/ year	6/26/2024
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

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

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

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

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

 | Data is current as of 4/18/2024

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Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
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 4/18/2024

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 Johnson & Johnson, the manufacturer of Stelara.
FYB202 *ustekinumab* SC injection	Formycon and Fresenius Kabi	STELARA®	Plaque psoriasis	BLA is under FDA review (BsUFA date: 9/30/2024)	No	• Would be the third STELARA® biosimilar, after WEZLANA™ and SELARSDI™.
SB17 *ustekinumab* SC injection	Samsung Bioepsis	STELARA®	Plaque psoriasis	BLA is under FDA review (BsUFA date: 11/1/2024)	No	• Would be a subsequent STELARA® biosimilar, after WEZLANA™ and SELARSDI™.
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 WEZLANA™ and SELARSDI™.
ENDOCRINOLOGY						
JUBBONTI® *denosumab-bbdz* SC injection	Sandoz	PROLIA®	Osteoporosis; increasing bone mass when receiving aromatase inhibitor therapy	FDA approval: 3/5/2024	No	• Is the first approved PROLIA® biosimilar product. • Due to ongoing patent litigation, Sandoz has not disclosed its expected launch timeline nor any pricing details.

 | Data is current as of 4/18/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
WYOST® *denosumab-bbdz* SC injection	Sandoz	XGEVA®	Skeletal-related complications of multiple myeloma and of bone metastases; giant cell tumor of the bone; hypercalcemia of malignancy	FDA approval: 3/5/2024	No	• Is the first approved XGEVA® biosimilar product. • Due to ongoing patent litigation, Sandoz has not disclosed its expected launch timeline nor any pricing details.
HEMATOLOGY						
ABP-959 *eculizumab* intravenous (IV) infusion	Amgen	SOLIRIS®	Paroxysmal nocturnal hemoglobinuria	BLA is under FDA review (BsUFA date: 2Q 2024)	No	• Would be the first approved SOLIRIS® biosimilar product.
IMMUNOLOGY						
SIMLANDI® *adalimumab-ryvk* SC injection	Alvotech and Teva	HUMIRA®	Rheumatoid arthritis (RA); polyarticular juvenile idiopathic arthritis (JIA); psoriatic arthritis; ankylosing spondylitis; Crohn's disease; ulcerative colitis (UC)	FDA approval: 2/23/2024	Yes	• First HUMIRA® biosimilar to be a high-concentration formulation that has been granted interchangeable status.
TYENNE® *tocilizumab-aazg* IV infusion and SC injection	Fresenius Kabi	ACTEMRA®	RA; JIA; giant cell arteritis	FDA approval: 3/5/2024	No	• Is the second biosimilar to ACTEMRA® after TOFIDENCE™, and the first to be approved with both IV and SC formulations.

 | Data is current as of 4/18/2024

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
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	BLA is under FDA review (BsUFA date: 6/15/2024) (interchangeability status)	Yes	• Would be a subsequent HUMIRA® interchangeable biosimilar product. • ABRILADA™, CYLTEZO® and SIMLANDI® have interchangeable status.
ONCOLOGY						
HLX02 *trastuzumab* IV infusion	Henlius Biotech	HERCEPTIN®	Breast cancer, gastric or gastroesophageal junction (GEJ) cancer	BLA is under FDA review (BsUFA date: 2Q 2024)	Yes	• Would be the sixth HERCEPTIN® biosimilar to be FDA-approved after HERZUMA®, KANJINTI®, OGIVRI®, ONTRUZANT®, and TRAZIMERA®.

 | Data is current as of 4/18/2024

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Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
DRL_RI *rituximab* IV 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 and Bausch + Lomb	LUCENTIS®	Wet age-related macular degeneration (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®.
YESAFILI™ *aflibercept* intraocular injection	Biocon	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 2Q 2024)	No	Could be the first FDA-approved biosimilar to EYLEA®.
FYB203 *aflibercept* intraocular injection	Formycon	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 6/29/2024)	No	Could be one of the first FDA-approved biosimilars to EYLEA®.
CTP42 *aflibercept* intraocular injection	Celltrion	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 6/29/2024)	No	Could be one of the first FDA-approved biosimilars to EYLEA®.

 | Data is current as of 4/18/2024

Generic Specialty Agents

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
mifeprisone	KORLYM®	Teva Pharmaceuticals	1/19/2024
deflazacort oral tablet	EMFLAZA®	Aurobindo	2/9/2024
Pipeline Agents			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**ANTICIPATED LAUNCH DATE**
nilotinib hydrochloride	TASIGNA®	Apotex	2Q 2024
lamivudine/raltegravir	DUTREBIS™	Undetermined	4/4/2024
edaravone intravenous	RADICAVA®	Undetermined	5/5/2024
dasatinib	SPRYCEL® (20, 50, 70, 80, 100, 140 mg)	Apotex	9/1/2024
octreotide acetate	SANDOSTATIN LAR®	Teva	2024
bendamustine oral solution	TREANDA®	Undetermined	2024
tolvaptan	JYNARQUE®	Lupin	4/23/2025

Includes generic agents with ≥ 50% launch probability

 | Data is current as of 4/18/2024

Glossary

Term	Definition
AA	alopecia areata
AADC	aromatic L-amino acid decarboxylase
ABR	annualized bleeding rate
AChR	acetylcholine receptor
AD	Alzheimer's disease
AMD	age-related macular degeneration
AML	acute myeloid leukemia
ARIA	amyloid-related imaging abnormalities
ARSA	arylsulfatase A
AS	ankylosing spondylitis
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
BTC	biliary tract cancer
CAR T-cell	chimeric antigen receptor T-cell
CD	Crohn's disease

Term	Definition
CDC	Centers for Disease Control and Prevention
CDR-SB	Clinical Dementia Rating-Sum of Boxes
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
cTTP	congenital thrombotic thrombocytopenic purpura
CV	cardiovascular
DEB	dystrophic epidermolysis bullosa
DED	dry eye disease
DLBCL	diffuse large B-cell lymphoma
DMD	Duchenne muscular dystrophy
EB	epidermolysis bullos
EGFR	epidermal growth factor receptor
ERT	enzyme replacement therapy
EVH	extravascular hemolysis
ET	essential thrombocythemia
FIX	factor IX

Term	Definition
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
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
ICER	Institute for Clinical and Economic Review
IV	intravenous
JAK1	Janus Kinase 1

 | Data is current as of 4/18/2024

CONTINUED

Glossary

Term	Definition
JAK2	Janus Kinase 2
JEB	junctional epidermolysis bullosa
JIA	juvenile idiopathic arthritis
LAD-I	leukocyte adhesion deficiency-I
LBCL	large B-cell lymphoma
LRTD	lower respiratory tract disease
MACE	major adverse cardiovascular events
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	nasopharyngeal carcinoma
NSAA	North Star Ambulatory Assessment
NSCLC	non-small cell lung cancer
NTF	neurotrophic factor
PAH	pulmonary arterial hypertension
PBC	primary biliary cholangitis
PD-L1	programmed death-ligand 1
PH1	primary hyperoxaluria type 1
pJIA	polyarticular juvenile idiopathic arthritis
pLGG	pediatric low-grade glioma
PPD	post-partum depression
PNH	paroxysmal nocturnal hemoglobinuria
PsA	psoriatic arthritis
PV	polycythemia vera
RA	rheumatoid arthritis
RDEB	recessive dystrophic epidermolysis bullosa
RRMM	relapsed or refractory multiple myeloma
SC	subcutaneous
SCD	sickle cell disease
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
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 4/18/2024

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