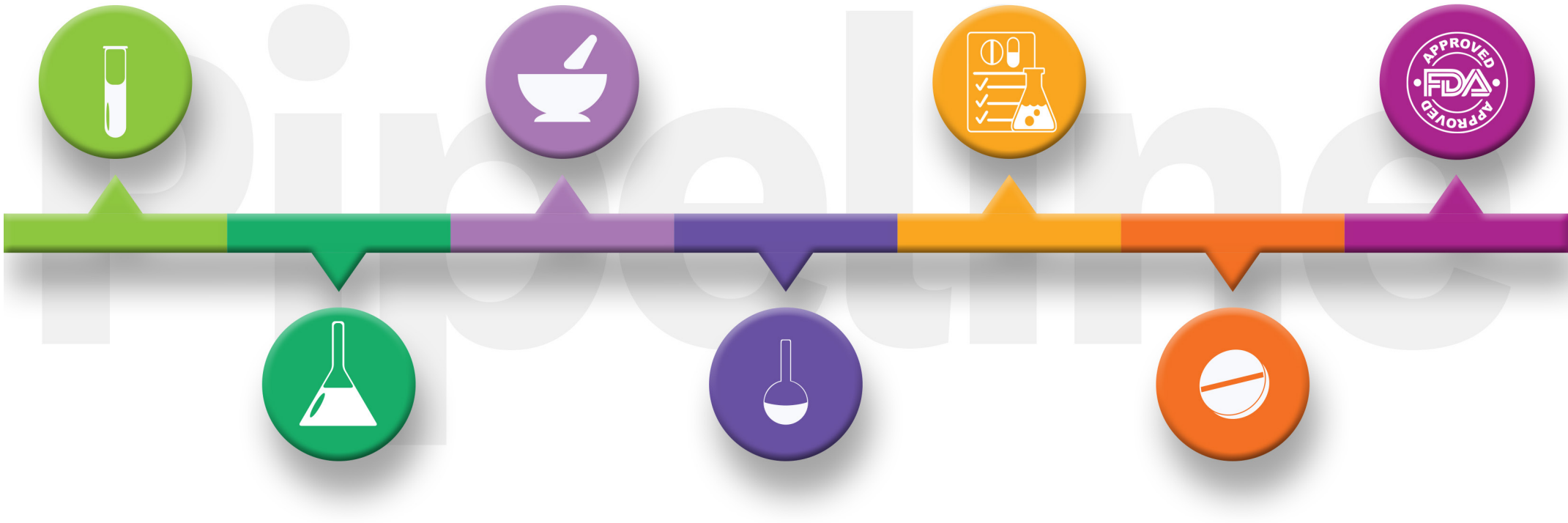

PIPELINE REPORT: OCTOBER 2024

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: **OCTOBER 2024**

Highlights

APPROVED: TECELRA *(afamitresgene autoleucel)* the first T-cell receptor immunotherapy that targets synovial sarcoma – a rare soft tissue sarcoma – becoming only the second cell therapy to be approved for a solid cancer.

APPROVED: AQNEURSA *(levacetylleucine)* and **MIPLYFFA** *(arimoclomol)* both FDA-approved for the treatment of neurological manifestations of Niemann-Pick disease type C, an ultra-rare genetic disorder with no previously approved therapies.

WITHDRAWN: OXBRYTA *(voxelotor)* previously FDA-approved for the treatment of sickle cell disease in adults and pediatric patients, has been voluntarily withdrawn from the market.

 | Data is current as of 10/21/2024

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
COAGULATION DISORDERS					
HYMPAVZI™ *marstacimab-hncq* SC injection	Pfizer	Hemophilia A or hemophilia B	10/11/2024	· Approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients ≥ 12 years of age with: hemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors, or hemophilia B (congenital factor IX deficiency) without factor IX inhibitors. · Once weekly SC injection. · For hemophilia A, will compete directly with HEMLIBRA® and factor VIII (FVIII) replacement therapy, while providing a chronic therapy alternative to ROCTAVIAN™ gene therapy. · For hemophilia B, will compete directly with FIX replacement therapy while providing a chronic therapy alternative to HEMGENIX® and BEQVEZ® gene therapies. · Projected impact: cost replacement of existing therapies.	$795,600/ year
DERMATOLOGY					
NEMLUVIO® *nemolizumab-ilto* SC injection Available through **AcariaHealth**	Galderma	Prurigo nodularis (PN)	8/12/2024	· Approved for the treatment of adults with PN. · Once monthly injection. · Would compete with DUPIXENT® which is a SC injection dosed every two weeks. · Projected impact: cost replacement of existing therapies.	Year 1: $55,120 Year 2 and thereafter: $50,880/year
EBGLYSS™ *lebrikizumab-lbkz* SC injection Available through **AcariaHealth**	Eli Lilly	Atopic dermatitis	9/13/2024	· Approved for the treatment of adult and pediatric patients ≥ 12 years of age who weigh ≥ 40 kg with moderate-to-severe atopic dermatitis whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable. · Ebglyss can be used with or without topical corticosteroids. · Multiple topical and injectable therapeutic alternatives (e.g., ADBRY®, DUPIXENT®) are available. · Projected impact: cost replacement of existing therapies.	Year 1: $63,000 Year 2 and thereafter: $42,000/year

 | Data is current as of 10/21/2024

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ENDOCRINOLOGY					
YORVIPATH® *palopegteriparatide* SC injection	Ascendis Pharma	Hypoparathyroidism	8/9/2024	· Approved for the treatment of adult patients with hypoparathyroidism. · Yorvipath is a long-acting prodrug of parathyroid hormone designed to restore PTH to physiologic levels, administered as a once-daily SC injection. · Will compete with NATPARA® until NATPARA® manufacturing ceases at the end of 2024. · Projected impact: cost replacement of existing therapies.	$285,000/ year
MIPLYFFA™ *arimoclomol* oral capsules	Zevra Therapeutics	Niemann-Pick disease type C (NPC)	9/20/2024	· Approved for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients ≥ 2 years of age. · NPC is an ultra-rare, progressive, genetic disorder with a prevalence of approximately one person per million in the U.S. · In a pivotal trial, patients taking Miplyffa plus miglustat for 52 weeks experienced a 2.2-point greater improvement in the rescored 4-domain NPC Clinical Severity Scale (R4DNPCCSS) score compared to patients who received placebo plus miglustat. A threshold for the minimal clinically important difference for the R4DNPCCSS has not been established. · There were insufficient data to determine the effectiveness of the use of Miplyffa without miglustat for the treatment of neurological manifestations in patients with NPC. · Will compete with Aqneursa which was also recently FDA-approved for NPC. · Projected impact: new cost in a very small population.	$480,000 - $1,272,000/ year, depending on dose ($1,020,000/ year for the typical-weight utilizer)

 | Data is current as of 10/21/2024

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
AQNEURSA™ *levacetylleucine* granules for oral suspension	IntraBio	NPC	9/24/2024	· Approved for the treatment of neurological manifestations of NPC in adults and pediatric patients weighing ≥ 15 kg. · In a pivotal trial patients taking Aqneursa for 12 weeks experienced a 0.4-point greater improvement on the functional Scale for the Assessment and Rating of Ataxia (fSARA) compared to those who received placebo. A threshold for the minimal clinically important difference for the fSARA has not been established. · Aqneursa does not require concomitant use with miglustat. · Will compete with the MIPLYFFA™ plus miglustat combination which was also recently FDA-approved for NPC. · Projected impact: new cost in a very small population.	$701,000/year at maximum weight-based dosing
GASTROENTEROLOGY					
LIVDELZI® *seladelpar* oral capsule	Gilead	Primary biliary cholangitis (PBC)	8/14/2024	· Approved 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. · Would compete with IQIRVO® and OCALIVA®, potentially with a lower rate of pruritus. · Projected impact: cost replacement of existing therapies.	$151,272/year

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
HEMATOLOGY					
OXBRYTA® *voxelotor* oral tablets and oral suspension	Pfizer	Sickle cell disease (SCD)	9/25/2024	• **Market withdrawal of a previously FDA-approved agent.** • OXBRYTA® was previously FDA-approved for the treatment of SCD in adults and pediatric patients ≥ 4 years of age. • Pfizer's decision to voluntarily withdraw OXBRYTA® from the market and to discontinue any ongoing clinical trials is based on the totality of clinical data that now indicates the overall benefit of OXBRYTA® no longer outweighs the risk in the approved sickle cell patient population. The data suggest an imbalance in vaso-occlusive crises and fatal events which require further assessment. • Remaining therapeutic alternatives include hydroxyurea, l-glutamine, and ADAKVEO®. • Projected impact: utilization will be replaced by other existing therapeutic alternatives.	Not applicable
IMMUNOLOGY					
LEQSELVI™ *deuruxolitinib* **oral tablet**	Sun Pharma	Alopecia areata (AA)	7/25/2024	• Approved for the treatment of adults with severe AA. • Will compete with OLUMIANT® and LITFULO™, two other agents with a similar mechanism of action that are FDA-approved for AA. • Projected impact: cost replacement of existing therapies.	Pending launch
NIKTIMVO™ *axatilimab-csfr* IV infusion	Incyte and Syndax	Chronic graft vs. host disease (cGVHD)	8/14/2024	• Approved for the treatment of cGVHD after failure of ≥ 2 prior lines of systemic therapy in adult and pediatric patients weighing ≥ 40 kg. • cGVHD is estimated to develop in approximately 40% of allogeneic hematopoietic stem cell transplantation (HSCT) recipients. • Will compete with other agents FDA-approved for cGVHD including, but not limited to, IMBRUVICA®, JAKAFI®, and REZUROCK®. • Projected impact: cost replacement of existing therapies.	Pending launch

 | Data is current as of 10/21/2024

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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	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 is 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

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ONCOLOGY					
TECELRA® *afamitresgene autoleucel* IV infusion	Adaptimmune	Synovial sarcoma	8/1/2024	**CELL THERAPY** · The FDA granted accelerated approval for the treatment of adults with unresectable or metastatic synovial sarcoma who have received prior chemotherapy, are HLA-A02:01P, -A02:02P, -A02:03P, or -A02:06P positive and whose tumor expresses the MAGE-A4 antigen as determined by FDA-approved or cleared companion diagnostic devices. · Approved with a Boxed Warning re: cytokine release syndrome. · The FDA approval of TECELRA® was based on the phase 2 SPEARHEAD-1 study which demonstrated an overall response rate (ORR) of 43.2%, including a complete response rate of 4.5% and a partial response rate of 38.6%. The median duration of response (DOR) observed in the trial was 6.0 months · Synovial sarcoma accounts for ~5% to 10% of all soft tissue sarcomas, with approximately 1,340 new cases identified per year. · Projected impact: new cost in a very small population.	$727,000/ one-time treatment
VORANIGO® *vorasidenib* oral tablets	Servier	Astrocytoma, oligodendroglioma	8/6/2024	· Approved for the treatment of adult and pediatric patients ≥12 years of age with Grade 2 astrocytoma or oligodendroglioma with a susceptible isocitrate dehydrogenase-1 (IDH1) or isocitrate dehydrogenase-2 (IDH2) mutation following surgery including biopsy, sub-total resection, or gross total resection. · Current therapeutic options include surgery, radiation, and/or chemotherapy. · Projected impact: cost replacement of existing therapies.	$485,219/year
LYMPHIR™ *denileukin diftitox-cxdl* IV infusion	Citius	Cutaneous T-cell lymphoma (CTCL)	8/7/2024	· Approved for the treatment of adult patients with relapsed or refractory (r/r) stage I-III CTCL after ≥ 1 prior systemic therapy. · LYMPHIR™ 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. · LYMPHIR™ maintains the same amino acid sequence but features improved purity and bioactivity over ONTAK®. · 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
LAZCLUZE™ + RYBREVANT® *lazertinib + amivantamab-vmjw* oral tablets + IV infusion	Janssen	Non-small cell lung cancer (NSCLC)	8/19/2024	· Approved for the first-line treatment of adult patients with locally advanced or metastatic NSCLC with epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 L858R substitution mutations, as detected by an FDA-approved test. · Will compete with TAGRISSO® and the TAGRISSO®-chemotherapy combination for the same indication. · Projected impact: cost replacement of existing therapies.	Year 1: $527,700 Year 2 and thereafter: $505,824/year
KEYTRUDA® *pembrolizumab* IV infusion	Merck	Malignant pleural mesothelioma (MPM)	9/17/2024	• **New indication for an existing agent.** · Approved for use in combination with with pemetrexed and platinum chemotherapy, as first-line treatment with adult patients with unresectable advanced or metastatic MPM. · Will compete with the combination of OPDIVO® plus YERVOY® which also is FDA-approved as a first-line treatment option for unresectable MPM. · Projected impact: cost replacement of existing therapies.	$204,072/year
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	NSCLC	10/3/2024	• **New indication for an existing agent.** · Approved for the treatment of adult patients with resectable (tumor ≥ 4 cm or node positive) NSCLC and no known EGFR mutations or ALK rearrangements, for neoadjuvant treatment in combination with platinum-doublet chemotherapy, followed by a single-agent OPDIVO® as adjuvant treatment after surgery. · Previously approved in the neoadjuvant setting; label expansion to include usage in the adjuvant setting. · Will compete with KEYTRUDA® which is FDA-approved for a similar indication. · Projected impact: cost replacement of existing therapies.	$190,793/year

 | Data is current as of 10/21/2024

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ITOVEBI™ *inavolisib* oral tablets Available through **AcariaHealth**	Genentech	Breast cancer	10/10/2024	· Approved 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 after completing adjuvant endocrine therapy. · PIQRAY® is FDA-approved for use in combination with fulvestrant for the same cancer type following progression on or after an endocrine-based regimen. · Projected impact: cost replacement of existing therapies.	$297,271/year
RESPIRATORY					
DUPIXENT® *dupilumab* subcutaneous injection Available through **AcariaHealth**	Regeneron and Sanofi	Chronic obstructive pulmonary disease (COPD)	9/27/2024	• **New indication for an existing agent.** · Approved for use as an add-on maintenance treatment of adult patients with inadequately controlled COPD and an eosinophilic phenotype. · In both of its pivotal trials DUPIXENT® demonstrated significant reductions in the annualized rate of moderate or severe COPD exacerbations compared to placebo when added to background maintenance therapy (29% and 34% reductions across the two trials, both $p < 0.001$). · Will be used as an adjunct to existing standard of care inhaled therapies. · Projected impact: incremental cost increase.	$49,442/year

 | Data is current as of 10/21/2024

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						

SPK-8011*
dirloctocogene 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 10/21/2024

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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	• 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 10/21/2024

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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
DERMATOLOGY						
delgocitinib topical cream	Leo Pharma	Chronic hand eczema (CHE)	Pan-Janus kinase (JAK) inhibitor	• Proposed for the treatment of adults with moderate to severe chronic hand eczema. • Current treatment options for CHE include steroids, calcineurin inhibitors, vitamin D derivatives, and emollients.	$15,000/year	7/23/2025
ENDOCRINOLOGY						
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.	$500,000/ year	11/28/2024
IONIS-APOCIII-LRx* *olezarsen* SC 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
DCCR* *diazoxide choline* extended-release oral tablet	Soleno Therapeutics	Prader-Willi syndrome (PWS)	Proglycemic agent	• Proposed for the treatment of adults and children ages 4 years and older with genetically confirmed PWS who have hyperphagia. • The hallmark symptom of PWS is hyperphagia, a chronic feeling of insatiable hunger that severely diminishes the quality of life for PWS patients and their families. Hyperphagia can lead to significant morbidities (e.g., stomach rupture, obesity, diabetes, cardiovascular disease) and mortality (e.g., choking, accidental death due to food seeking behavior). • There are currently no FDA-approved therapies for hyperphagia associated with PWS.	$500,000/ year	12/27/2024

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

 | Data is current as of 10/21/2024

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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
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.	$500,000/ year	12/29/2024 (oral capsule) 12/30/2024 (oral solution)
MTP131* *elamipretide* SC injection	Stealth BioTherapeutics	Barth syndrome	Mitochondrial cardiolipin stabilizer	• Barth syndrome is an ultra-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. • On 10/10/2024, the FDA's Cardiovascular and Renal Drugs Advisory Committee voted 10-6 that elamipretide is effective for Barth syndrome, and cited the urgent unmet need.	$850,000/ year	1/29/2025
PTC923 *sepiapterin* oral therapy	PTC Therapeutics	Phenylketonuria (PKU)	Tetrahydro-biopterin precursor	• Proposed for the treatment of pediatric and adult patients with PKU	$125,000/ year	2Q 2025

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

 | Data is current as of 10/21/2024

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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						
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
IMMUNOLOGY						
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
KVD900 *sebetralstat* oral therapy	KalVista Pharmaceuticals	Hereditary angioedema (HAE)	Kallikrein inhibitor	• Proposed for the on-demand treatment of HAE attacks in adults and pediatric patients aged > 12 years. • Would compete with other HAE therapies used for on-demand treatment such as FIRAZYR® (now generic) and RUCONEST®. • If approved, sebetralstat would be the first oral, on-demand therapy for people living with HAE.	$350,000/ year	6/17/2025

*** 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
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

 | Data is current as of 10/21/2024

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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						

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.**

Upcoming Specialty Products

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

MCLA-128
zenocutuzumab
IV 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

IMAB362 *zolbetuximab* IV 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
ZW25 *zanidatamab* IV 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
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	12/26/2024

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

 | Data is current as of 10/21/2024

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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
cosibelimab IV infusion	Checkpoint Therapeutics	Cutaneous squamous cell carcinoma (cSCC)	Anti-PD-L1 antibody	• Proposed for the treatment of patients with metastatic cSCC or locally advanced cSCC who are not candidates for curative surgery or radiation.	$250,000/ year	12/28/2024
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 ≥ 2 years of age two years of age 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
treosulfan IV 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	1/30/2025
DCC-3014 *vimseltinib* oral therapy	Ono Pharmaceuticals	Tenosynovial giant cell tumor (TGCT)	CSF1R inhibitor	• Proposed for the treatment of patients with TGCT not amenable to surgery. • Would compete with TURALIO® for the same indication.	$300,000/ year	2/17/2025
PD-0325901 *mirdametinib* oral therapy	SpringWorks Therapeutics	Neurofibromatosis type 1- associated plexiform neurofibromas (NF1-PN)	Mitogen-activated protein kinase 1 and 2 (MEK 1/2) inhibitor	• Proposed for the treatment of NF1-PN in patients ≥2 years of age. • Would compete with KOSELUGO® which is also FDA-approved for NF1-PN.	$250,000/ year	2/28/2025

Expected to cost ≥ $500,000 per member.

 | Data is current as of 10/21/2024

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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
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	HCC	PD-1 inhibitor	• **New indication for an existing agent.** • Proposed in combination with YERVOY® for the first-line treatment of unresectable HCC. • OPDIVO® is currently approved in combination with YERVOY® for the treatment of HCC in patients previously treated with sorafenib.	$190,793/ year	4/21/2025
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
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	4Q2024

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

 | Data is current as of 10/21/2024

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
DERMATOLOGY						
OTULFI™ *ustekinumab-aauz* SC injection	Formycon and Fresenius Kabi	STELARA®	Adults with plaque psoriasis (PsO), psoriatic arthritis (PsA), Crohn's disease (CD), or ulcerative colitis (UC); Pediatric patients 6 years and older with PsO or PsA	FDA approval: 9/27/2024	No	• Is the fourth STELARA® biosimilar, after PYZCHIVA®, SELARSDI™, and WEZLANA™. • In accordance with the patent settlement between Fresenius Kabi, Formycon and Johnson & Johnson, Fresenius Kabi has the right to market OTULFI™ in the US no later than February 22, 2025.
IMULDOSA™ *ustekinumab-srlf* SC injection	Accord BioPharma	STELARA®	Adults with PsO, PsA, CD, UC; Pediatric patients 6 years and older with PsO or PsA	FDA approval: 10/10/2024	No	• Is the fifth STELARA® biosimilar, after OTULFI™, PYZCHIVA®, SELARSDI™, and WEZLANA™. • A commercial launch date for IMULDOSA™ has not been announced.
BAT2206 *ustekinumab* SC injection	Bio-Thera Solutions	STELARA®	PsO	BLA is under FDA review (BsUFA date: 2Q 2025)	No	• Would be the sixth STELARA® biosimilar, after IMULDOSA™, OTULFI™, PYZCHIVA®, SELARSDI™, and WEZLANA™.

 | Data is current as of 10/21/2024

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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						
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.
IMMUNOLOGY						
AMJEVITA™ *adalimumab-atto* SC injection	Amgen	HUMIRA®	RA; JIA; PsA; ankylosing spondylitis (AS); CD; UC; PsO; hidradenitis suppurativa (HS); uveitis	FDA approval of interchangeable status: 8/20/2024	Yes	• ABRILADA™, CYLTEZO®, HYRIMOZ®, and SIMLANDI® also have interchangeable status. • Interchangeable designation was granted to only the following forms/strengths of AMJEVITA™: prefilled autoinjector 40 mg/0.8 mL, prefilled syringes 40 mg/0.8 mL, 20 mg/0.4 mL, 10 mg/0.2 mL.

 | Data is current as of 10/21/2024

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Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
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®.
ONCOLOGY						
DRL_RI *rituximab* IV infusion	Dr. Reddy's Laboratories	RITUXAN®	Non-Hodgkin's lymphoma, RA	BLA is under FDA review (BsUFA date: 4Q 2024)	Yes	· Would be the fourth RITUXAN® biosimilar to be FDA-approved after RIABNI™, RUXIENCE®, and TRUXIMA®.
TX-05 *trastuzumab* IV infusion	Tanvex BioPharma	HERCEPTIN®	Breast cancer, gastric cancer	BLA is under FDA review (BsUFA date: 1/6/2025)	Yes	· Would be the seventh HERCEPTIN® biosimilar, after HERCESSI®, HERZUMA®, KANJINTI®, OGIVRI®, ONTRUZANT®, and TRAZIMERA™.
OPHTHALMOLOGY						
ENZEEVU™ *aflibercept-abzv* intraocular injection	Sandoz	EYLEA®	Wet age-related macular degeneration (AMD)	FDA approval: 8/9/2024	No	· Is the fourth EYLEA® biosimilar, after AHZANTIVE®, OPUVIZ™, and YESAFILI™ .
PAVBLU™ *aflibercept-ayyh* intraocular injection	Amgen	EYLEA®	Wet AMD, macular edema following retinal vein occlusion (RVO), diabetic macular edema (DME), and diabetic retinopathy (DR)	FDA approval: 8/23/2024	No	· Is the fifth EYLEA® biosimilar, after AHZANTIVE®, ENZEEVU™, OPUVIZ™, and YESAFILI™ .
CTP42 *aflibercept* intraocular injection	Celltrion	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 4Q 2024)	No	· Would be a subsequent EYLEA® biosimilar, after AHZANTIVE®, OPUVIZ™, and YESAFILI™ .

 | Data is current as of 10/21/2024

Generic Specialty Agents

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
l-glutamine	ENDARI®	Novitium	7/15/2024
tacrolimus	ASTAGRAF XL®	Chengdu Suncadia Medicines	7/17/2024
dasatinib	SPRYCEL® (20, 50, 70, 80, 100, 140 mg)	Apotex	9/4/2024
octreotide acetate	SANDOSTATIN LAR®	Teva	10/1/2024
Pipeline Agents*			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**ANTICIPATED LAUNCH DATE**
nilotinib	TASIGNA®	Apotex, Teva	2H 2024
bendamustine oral solution	TREANDA®	Fresenius Kabi	2024
tolvaptan	JYNARQUE®	Lupin	4/23/2025
glycerol phenylbutyrate	RAVICTI®	Par/Endo	7/1/2025
rilpivirine hydrochloride	EDURANT®	Strides	10/22/2025
macitentan	OPSUMIT®	Alembic; Amneal; Apotex; Aurobindo; Laurus Labs; MSN Laboratories; Mylan/Viatris; Seasons Biotechnology (Taizhou); Sun; Teva; Zydus	12/5/2025

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

 | Data is current as of 10/21/2024

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 10/21/2024

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
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
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 10/21/2024

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 10/21/2024

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