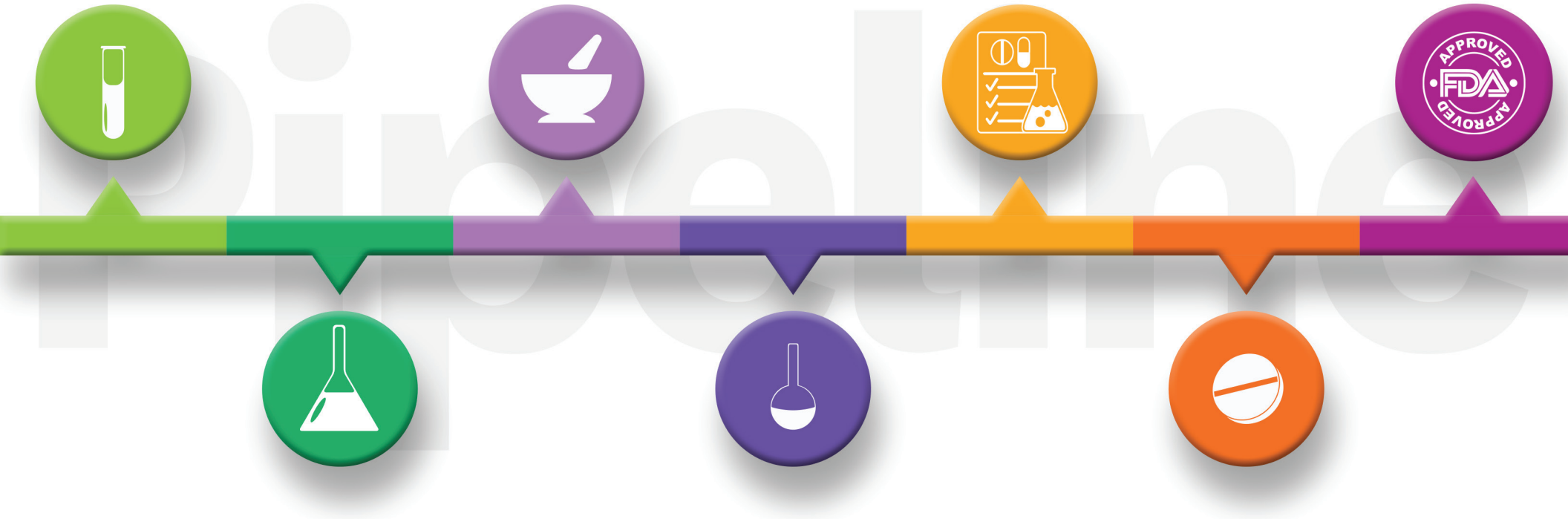

PIPELINE REPORT: JANUARY 2025

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.

PIPELINE REPORT: **JANUARY 2025**

Highlights

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APPROVED: TRYNGOLZA *(olezarsen)* is the first drug approved to treat familial chylomicronemia syndrome (FCS)

APPROVED: CRENESSITY *(crinecerfont)* for congenital adrenal hyperplasia (CAH)

APPROVED: ZEPBOUND *(tirzepatide)* for obstructive sleep apnea

 | Data is current as of 1/25/2025

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
CARDIOVASCULAR DISEASE					
ATTRUBY™ *acoramidis* oral tablet	BridgeBio Pharmaceuticals	Cardiomyopathy	11/22/2024	· Approved for the treatment of cardiomyopathy of wild-type or variant transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular death and cardiovascular-related hospitalization. · Will compete with VYNDAQEL® and VYNDAMAX® which are FDA-approved for the same indication. · Projected impact: cost replacement of existing therapies.	$243,869/year
COAGULATION DISORDERS					
HYMPAVZI™ *marstacimab-hncq* SC injection Available through **AcariaHealth**	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 (FVIII) inhibitors, or hemophilia B (congenital factor IX deficiency) without factor IX (FIX) inhibitors. · Once weekly SC injection. · For hemophilia A, will compete directly with HEMLIBRA® and 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

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ALHEMO® *concizumab-mtci* SC injection	Novo Nordisk	Hemophilia A or hemophilia B	12/20/2024	• 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 FVIII deficiency) with FVIII inhibitors or hemophilia B (congenital FIX deficiency) with FIX inhibitors. • Once daily SC injection. • For hemophilia A, will compete directly with HEMLIBRA®, bypassing agents (e.g., FEIBA, NovoSeven RT, SevenFact), and immune tolerance induction therapy with FVIII replacement products. • For hemophilia B, will compete directly with bypassing agents (e.g., FEIBA, NovoSeven RT, SevenFact) and immune tolerance induction therapy with FIX replacement products. • Projected impact: cost replacement of existing therapies.	Pending launch
ENDOCRINOLOGY					
CRENESSITY™ *crinecerfont* oral capsule and oral solution	Neurocrine Biosciences	Congenital adrenal hyperplasia (CAH)	12/13/2024	• Approved for use as adjunctive treatment to glucocorticoid replacement to control androgens in adults and pediatric patients ≥ 4 years of age 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. • Projected impact: incremental cost increase in a small population.	$466,382/year

 | Data is current as of 1/25/2025

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
TRYNGOLZA™ *olezarsen* SC injection	Ionis Pharmaceuticals	Familial chylomicronemia syndrome (FCS)	12/19/2024	• Approved for use as an adjunct to diet to reduce triglycerides in adults with FCS. • FCS is a rare, genetic disease characterized by extremely elevated triglyceride levels, impacting an estimated 1-2 people per million worldwide. • TRYNGOLZA™ is the first FDA-approved therapy for the treatment of FCS. Patients previously relied solely on nutrition management through extremely restrictive diets. • Projected impact: new cost in a very small population.	$595,000/ year
IMMUNOLOGY					
RYONCIL® *remestemcel-L-rknd* IV injection	Mesoblast	Steroid-refractory acute graft vs. host disease (SR-aGvHD)	12/18/2024	**CELL THERAPY** • Approved for the treatment of SR-aGvHD in pediatric patients ≥ 2 months of age. • RYONCIL® is 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. • Projected impact: children ≥ 12 years of age - cost increase in a small population; children ≥ 12 years of age - 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
NEUROLOGY					
KEBILIDI™ *eladocagene exuparvovec-tneq* intraputamenal injection	PTC Therapeutics	Aromatic L-amino acid decarboxylase (AADC) deficiency	11/13/2024	**GENE THERAPY** · Approved for the treatment of adult and pediatric patients with AADC deficiency. · First FDA-approved therapy for AADC deficiency which is an ultra-rare enzyme deficiency disorder with an estimated prevalence of < 5,000 patients worldwide. · Five-year follow-up results from a clinical trial show that motor function improvements after KEBILIDI therapy were sustained, demonstrating a durable treatment effect. · Projected cost impact: cost increase in a very small population.	$3.95 million/ one-time treatment
ONCOLOGY					
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	Non-small cell lung cancer (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

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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
VYLOY® *zolbetuximab-clzb* IV infusion	Astellas	Gastric or gastroesophageal junction (GEJ) adenocarcinoma	10/18/2024	· Approved for use in combination with fluoropyrimidine- and platinum-containing chemotherapy for the first-line treatment of adults with locally advanced unresectable or metastatic HER2-negative gastric or GEJ adenocarcinoma whose tumors are CLDN18.2-positive as determined by an FDA-approved test. · Projected impact: cost replacement of existing therapies.	$332,800/year
AUCATZYL® *obecabtagene autoleucel* IV infusion	Autolus Therapeutics	Acute lymphoblastic leukemia (ALL)	11/8/2024	**CAR T-CELL THERAPY** · Approved for the treatment of adults with relapsed or refractory B-cell precursor ALL. · Administered as two doses given 10 days apart. · Has overlapping indications with TECARTUS® and KYMRIAH®, with a favorable tolerability profile over these two alternatives. · All three agents have the same Boxed Warnings re: cytokine release syndrome, neurologic toxicities, and secondary hematological malignancies. · Projected impact: cost replacement of existing therapies.	$525,000/ one-time treatment

 | Data is current as of 1/25/2025

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
REVUFORJ® *revumenib* oral tablets	Syndax Pharmaceuticals	Acute leukemia	11/15/2024	• Approved for the treatment of relapsed or refractory acute leukemia with a lysine methyltransferase 2A gene (*KMT2A*) translocation in adult and pediatric patients 1 year and older. • Prescribing Information includes a Boxed Warning re: differentiation syndrome. • *KMT2A* gene rearrangements give rise to *KMT2Ar* acute leukemia known to have a poor prognosis, with < 25% of adult patients surviving past five years. • Projected impact: new cost in a small population.	$474,000/ year
ZIIHERA® *zanidatamab-hrii* IV infusion	Jazz Pharmaceuticals & Zymeworks	Biliary tract cancer (BTC)	11/20/2024	• Approved for the treatment of adults with previously treated, unresectable or metastatic HER-2 positive (IHC 3+) BTC, as detected by an FDA-approved test. • Prescribing Information includes a Boxed Warning re: embryo-fetal toxicity. • Projected impact: cost replacement of existing therapies.	$461,500/year
BIZENGRI® *zenocutuzumab-zbco* IV infusion	Merus	NSCLC; pancreatic cancer	12/4/2024	• Approved for the treatment of adults with advanced, unresectable or metastatic pancreatic adenocarcinoma or NSCLC harboring a neurgulin 1 (*NRG1*) gene fusion with disease progression on or after systemic therapy. • *NRG1* fusions are rare, occurring in < 1% of solid tumors. • Prescribing Information includes a Boxed Warning re: embryo-fetal toxicity. • Projected impact: new cost in a small population.	$308,750/year
UNLOXCYT™ *cosibelimab-ipdl* IV infusion	Checkpoint Therapeutics	Cutaneous squamous cell carcinoma (cSCC)	12/13/2024	• Approved for the treatment of adults with metastatic cSCC or locally advanced cSCC who are not candidates for curative surgery or curative radiation. • Projected impact: cost replacement of existing therapies.	Pending launch

 | Data is current as of 1/25/2025

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ENSACOVE™ *ensartinib* oral capsules	Xcovery	NSCLC	12/18/2024	· Approved for the treatment of adult patients with anaplastic lymphoma kinase (*ALK*)-positive locally advanced or metastatic NSCLC who have not previously received an *ALK*-inhibitor. · Will compete with multiple other alternatives available as first-line therapies for advanced disease, including: ALECTINIB, BRIGATINIB, CERITINIB, CRIZOTINIB, and LORLATINIB. · Projected impact: cost replacement of existing therapies.	Pending launch
OPDIVO QVANTIG™ *nivolumab/ hyaluronidase-nvhy* SC infusion	Bristol Myers Squibb	Multiple solid tumors for which IV OPDIVO is also FDA-approved	12/27/2024	· Approved for most of the previously approved adult, solid tumor OPDIVO indications as monotherapy, monotherapy maintenance following completion of OPDIVO plus YERVOY combination therapy, or in combination with chemotherapy or cabozantinib. · OPDIVO QVANTIG is administered as a single SC injection over 3-5 minutes vs. 30 minutes for the Opdivo IV formulation. · Projected impact: cost replacement of existing therapies	$202,470/year
RESPIRATORY					
ALYFTREK™ *vanzacaftor/ tezacaftor/ deutivacaftor* oral tablet	Vertex	Cystic fibrosis (CF)	12/20/2024	· Approved for the treatment of CF in patients aged ≥ 6 years who have at least one *F508del* mutation or another responsive mutation in the *CFTR* gene. · Once-daily dosing. · FDA approved with a Boxed Warning re: drug-induced liver injury and liver failure. · Will compete with TRIKAFTA®. · Projected impact: cost replacement of existing therapies.	$370,269/year
SURGERY					
SYMVESS™ *acellular tissue engineered vessel-tyod* surgical implant	Humacyte	Vascular trauma	12/19/2024	· Approved for use in adults as a vascular conduit for extremity arterial injury when urgent revascularization is needed to avoid imminent limb loss, and autologous vein graft is not feasible. · Off-the-shelf, bioengineered tissue; infection-resistant, universally implantable conduit for use in vascular repair. · Projected impact: cost replacement of existing therapy.	Pending launch

 | Data is current as of 1/25/2025

Upcoming Specialty Products

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
CARDIOVASCULAR DISEASE						
CK-3773274 *aficamten* oral therapy	Cytokinetics, Inc.	Obstructive hypertrophic cardiomyopathy (oHCM)	Cardiac myosin inhibitor	• Proposed for the treatment of symptomatic oHCM. • Would compete with CAMZYOS®, which is FDA-approved for the same indication.	$100,000/ year	9/26/2025
COAGULATION DISORDERS						
fitusiran (S) SC injection	Alnylam/Sanofi	Hemophilia A or hemophilia B	Antithrombin III-targeting small interfering ribonucleic acid (siRNA) oligonucleotide	• Proposed for the treatment of hemophilia A or B in adults and adolescents with or without inhibitors. • Once-monthly self-administered SC injection. • Will enter a crowded market of available alternatives for both hemophilia A and hemophilia B, including recently FDA-approved products ALHEMO® and HYMPAVZI™.	$750,000/ year	3/28/2025

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

 | Data is current as of 1/25/2025

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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 (S)
giroctocogene fitelparvovec
IV infusion
	Sangamo Therapeutics	Hemophilia A	Gene therapy	• 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 annualized bleeding rate (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 significant reduction in mean total ABR compared to the pre-infusion period (1.24 vs 4.73; p = 0.0040).
				  • Secondary endpoints were met and demonstrated superiority compared to FVIII prophylaxis: 84% of participants (Ps) 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. Among all dosed Ps, one (1.3%) returned to FVIII prophylaxis post-SB-525 infusion.
				  • Transient elevated FVIII levels ≥ 150% were observed in 49.3% of dosed Ps, with no impact on efficacy and safety results. Serious adverse events were reported in 15 Ps (20%), including 13 events reported by 10 Ps (13.3%) assessed as related to treatment.
				• Pfizer terminated its co-development agreement with Sangamo for this product. Sangamo plans to explore options to continue to advance the program.
				• Would compete with ROCTAVIAN™ gene therapy for the same indication.
					$2-3 million/ one-time treatment	2025

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

 | Data is current as of 1/25/2025

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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						
Pz-cel $ *prademagene zamikeracel* epidermal sheet	Abeona Therapeutics	Recessive dystrophic epidermolysis bullosa (RDEB)	Gene therapy	**GENE THERAPY** • Proposed for the treatment of RDEB in patients > 6 years of age. • Pz-cel is produced as autologous, COL7A1 gene-corrected epidermal sheets which are laid over RDEB wounds to promote wound healing and pain reduction. • VYJUVEK® is another gene therapy that is also FDA-approved for RDEB, but which is applied topically weekly until wound closure.	$2.5-3.5 million/ one-time treatment The development of new RDEB lesions in different areas of the body would require additional courses of therapy.	4/29/2025
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

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

 | Data is current as of 1/25/2025

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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						
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.	$750,000/ year	4/29/2025
DCCR $ *diazoxide choline* extended-release oral tablet	Soleno Therapeutics	Prader-Willi syndrome (PWS)	Proglycemic agent	• Proposed for the treatment of adults and children ≥ 4 years of age 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	3/27/2025

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

 | Data is current as of 1/25/2025

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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
AEB1102 Ⓢ *pegzilarginase* IV infusion	Immedica	Arginase I deficiency	Enzyme replacement therapy	· Proposed for the treatment of arginase I deficiency, which is one of the eight urea cycle disorder subtypes. · Arginase I deficiency has a global prevalence of ~1 in every 1,000,000 people and is identifiable via newborn screening. · Pegzilarginase would be the first FDA-approved treatment for this condition.	$500,000/ year	4/5/2025
CUTX-101 Ⓢ *copper histidinate* SC injection	Fortress Biotech, Sentynl Therapeutics	Menkes disease	Copper replacement therapy	· Menkes disease is a rare X-linked pediatric disease caused by gene mutations of copper transporter ATP7A. · There is currently no FDA-approved treatment specifically for Menkes disease and its variants; however, daily injections of copper supplements are currently used as an off-label therapy, along with supportive therapies. · Most Menkes disease patients do not survive past age 3 without treatment.	$500,000/ year	6/30/2025
PTC923 *sepiapterin* oral therapy	PTC Therapeutics	Phenylketonuria (PKU)	Tetra-hydrobiopterin precursor	· Proposed for the treatment of pediatric and adult patients with PKU. · Would compete with KUVAN® (available generically as sapropterin) for the same indication.	$125,000/ year	7/30/2025
CRN00808 *paltusotine* oral capsule	Crinetics Pharmaceuticals	Acromegaly	Somatostatin type 2 receptor agonist	· Proposed for the treatment and long-term maintenance therapy of acromegaly in adults. · Once-daily oral therapy. · Would compete with injectable somatostatin analogs (e.g., SOMATULINE® DEPOT, SANDOSTATIN®, SIGNIFOR) and twice-daily oral MYCAPSSA®.	$175,000/ year	9/25/2025

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

 | Data is current as of 1/25/2025

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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						
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
IONIS-PKK-LRX (S) *donidalorsen* SC injection	Ionis Pharmaceuticals	Hereditary angioedema (HAE)	Prekallikrein-targeting antisense oligonucleotide	· Proposed for the prophylaxis of acute attacks of HAE in adult and pediatric patients ≥ 12 years of age. · Would compete with existing HAE prophylactic agents including, CINRYZE®, ORLADEYO® and TAKHZYRO®.	$650,000/ year	8/21/2025
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
TRANSLARNA (S) *ataluren* oral therapy	PTC Therapeutics	Duchenne muscular dystrophy (DMD)	Protein restoration therapy	· Proposed for the treatment of nonsense mutation DMD (nmDMD). · It is estimated that nonsense mutations account for approximately 13% of DMD cases.	$750,000/ year	4/30/2025

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

 | Data is current as of 1/25/2025

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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
NEUROMUSCULAR CONDITIONS						
M281 *nipocalimab* IV infusion	Johnson & Johnson	Generalized myasthenia gravis (gMG)	Anti-FcRn monoclonal antibody	· Proposed for the treatment of antibody positive (anti-AChR, anti-MuSK, anti-LRP4) patients with gMG. · Would compete with VYVGART® and VYVGART HYTRULO® in AChR antibody-positive patients, and with RYSTIGGO® in AChR- or MuSK antibody-positive patients. · Antibodies specific for LRP4 occur in up to 5% of patients with MG.	$300,000/ year	8/9/2025
ONCOLOGY						
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	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
rivoceranib + camrelizumab oral therapy and IV infusion	Elevar Therapeutics	Hepatocellular carcinoma (HCC)	Tyrosine kinase inhibitor + PD-1 inhibitor	· Proposed for use as first-line treatment for unresectable or metastatic HCC.	$400,000/ year	3/20/2025

 | Data is current as of 1/25/2025

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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	Hepatocellular carcinoma (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
taletrectinib oral therapy	Nuvation Bio	Non-small cell lung cancer (NSCLC)	ROS1 inhibitor	• Proposed for the treatment of adult patients with advanced or metastatic ROS1-positive NSCLC who are ROS1 tyrosine kinase inhibitor (TKI) treatment naïve or previously treated with crizotinib.	$300,000/ year	6/23/2025
avutometinib + defactinib oral therapy	Verastem Oncology	Ovarian cancer	avutometinib: MEK 1/2 inhibitor defactinib: focal adhesion kinase (FAK) inhibitor	• Proposed for combination use for the treatment of patients with KRAS mutant recurrent low-grade serous ovarian cancer who received at least one prior systemic therapy.	$350,000/ year	6/30/2025
DZD9008 *sunvozertinib* oral therapy	Dizal	Non-small cell lung cancer (NSCLC)	EGFR inhibitor	• Proposed for the treatment of patients with locally advanced or metastatic NSCLC with epidermal growth factor receptor (EGFR) exon 20 insertion mutations (exon20ins), as detected by an FDA-approved test, whose disease has progressed on or after platinum-based chemotherapy. • RYBREVANT® intravenous infusion is FDA-approved for use as a single agent for the same indication.	$350,000/ year	7/7/2025

 | Data is current as of 1/25/2025

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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
BLENREP *belantamab mafodotin-blmf* IV infusion	GSK	Multiple myeloma (MM)	Anti-B-cell maturation antigen (BCMA) antibody-drug conjugate (ADC)	· Proposed market re-entry for use in combinations with bortezomib plus dexamethasone or with pomalidomide plus dexamethasone for the treatment of patients with MM who have received at least one prior line of therapy. · Was previously removed from the market for use as monotherapy for relapsed/refractory MM as 5th-line or later therapy, after failing to demonstrate superiority over the combination of Pomalyst plus dexamethasone in a Phase III confirmatory study. · Regulatory re-submission is supported by the results of the Phase III DREAMM-7 and DREAMM-8 trials showing statistically significant efficacy, including improvement in overall survival in DREAMM-7.	$350,000/ year	7/23/2025
DATO-DXd *datopotamab deruxtecan* IV infusion	AstraZeneca	Non-small cell lung cancer (NSCLC)	TROP2-directed DXd antibody drug conjugate	· Proposed for the treatment of adult patients with locally advanced or metastatic epidermal growth factor receptor-mutated (EGFRm) NSCLC who have received prior systemic therapies, including an EGFR-directed therapy.	$350,000/ year	3Q 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	3/18/2025

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

 | Data is current as of 1/25/2025

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
DERMATOLOGY						
IMULDOSA™ *ustekinumab-srlf* SC injection	Accord BioPharma	STELARA®	Adults with plaque psoriasis (PsO), psoriatic arthritis (PsA), Crohn's disease (CD), ulcerative colitis (UC); Pediatric patients ≥6 years of age with PsO or PsA	FDA approval: 10/10/2024	No	• Is the fifth STELARA® biosimilar, after OTULFI®, PYZCHIVA®, SELARSDI®, and WEZLANA®. • A commercial launch of IMULDOSA is anticipated in 1H 2025.
YESINTEK™ *ustekinumab-kfce* SC injection	Biocon Biologics	STELARA®	Adults with PsO, PsA, CD, UC; Pediatric patients ≥6 years of age with PsO or PsA	FDA approval: 11/29/2024	No	• Is the sixth STELARA biosimilar, after IMULDOSA™, OTULFI, PYZCHIVA, SELARSDI, and WEZLANA. • YESINTEK™ is expected to launch in the U.S. no later than February 22, 2025.
STEQEYMA® *ustekinumab-stba* SC injection	Celltrion	STELARA®	Adults with PsO, PsA, CD, UC; Pediatric patients ≥6 years of age with PsO or PsA	FDA approval: 12/17/2024	No	• Is the seventh STELARA biosimilar, after IMULDOSA, OTULFI, PYZCHIVA, SELARSDI, WEZLANA, and YESINTEK™. • STEQEYMA is expected to launch in the U.S. in February 2025.
BAT2206 *ustekinumab* SC injection	Bio-Thera Solutions	STELARA®	PsO	BLA is under FDA review (BsUFA date: 2Q 2025)	No	• Would be the eighth STELARA biosimilar, after IMULDOSA, OTULFI, PYZCHIVA, SELARSDI, STEQEYMA, WEZLANA, and YESINTEK.

 | Data is current as of 1/25/2025

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®.
HLX14 *denosumab* SC injection	Shanghai Henlius Biotech, Inc.	PROLIA®	Postmenopausal osteoporosis	BLA is under FDA review (BsUFA date:8/30/2025)	No	• Would be a subsequent denosumab biosimilar, after JUBBONTI and WYOST.
RGB-14 *denosumab* SC injection	Hikma	XGEVA®	Osteoporosis and fractures due to bone metastasis	BLA is under FDA review (BsUFA date: 4Q 2025)	No	• Would be a subsequent denosumab biosimilar, after JUBBONTI and WYOST.
TVB-009P *denosumab* SC injection	Teva	PROLIA®	Osteoporosis	BLA is under FDA review (BsUFA date: 2H 2025)	No	• Would be a subsequent denosumab biosimilar, after JUBBONTI and WYOST.
IMMUNOLOGY						
CT-P39 *omalizumab* SC injectio	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 1/25/2025

CONTINUED

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
ONCOLOGY						
TX-05 *trastuzumab* IV infusion	Tanvex BioPharma	HERCEPTIN®	Breast cancer, gastric cancer	BLA is under FDA review (BsUFA date: 1Q 2025)	Yes	• Would be the seventh HERCEPTIN® biosimilar, after HERCESSI®, HERZUMA®, KANJINTI®, OGIVRI®, ONTRUZANT®, and TRAZIMERA™.
OPHTHALMOLOGY						
CTP42 *aflibercept* intraocular injection	Celltrion	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 1Q 2025)	No	• Would be a subsequent EYLEA® biosimilar, after AHZANTIVE®, OPUVIZ™, and YESAFILI™ .

 | Data is current as of 1/25/2025

Generic Specialty Agents

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
octreotide acetate	SANDOSTATIN LAR®	Teva	10/1/2024
Pipeline Agents*			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**ANTICIPATED LAUNCH DATE**
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	4Q 2025

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

 | Data is current as of 1/25/2025

Glossary

Term	Definition
AADC	aromatic L-amino acid decarboxylase
ABR	annualized bleeding rate
ADC	antibody-drug conjugate
AHI	apnea-hypopnea index
ALL	acute lymphoblastic leukemia
allo-HSCT	allogeneic hematopoietic stem cell transplantation
AMD	age-related macular degeneration
ATTR-CM	transthyretin amyloidosis cardiomyopathy
BCMA	B-cell maturation antigen
BLA	biologics license application
BsUFA	Biosimilar User Fee Act
BTC	biliary tract cancer
CAH	congenital adrenal hyperplasia
CAR T-cell	chimeric antigen receptor T-cell
CD	Crohn's disease
CDC	Centers for Disease Control and Prevention
CF	cystic fibrosis
CHE	chronic hand eczema
CKD	chronic kidney disease
CMS	Centers for Medicare & Medicaid Services

Term	Definition
CNTF	ciliary neurotrophic factor
cSCC	cutaneous squamous cell carcinoma
DED	dry eye disease
DMD	Duchenne muscular dystrophy
EBV	Epstein Barr virus
EGFR	epidermal growth factor receptor
ERT	enzyme replacement therapy
FAK	focal adhesion kinase
FCS	familial chylomicronemia syndrome
FDA	Food and Drug Administration
FIX	factor IX
FVIII	factor VIII
GEJ	gastroesophageal junction
GLP-1	glucagon-like peptide-1
HAE	hereditary angioedema
HCC	hepatocellular carcinoma
HER	human epidermal growth factor receptor
HR	hormone receptor
HSCT	hematopoietic stem cell transplantation
IV	intravenous

Term	Definition
JAK	Janus Kinase
LDH	lumbar disc herniation
MEK 1/2	mitogen-activated protein kinase 1 and 2
MM	multiple myeloma
NF1-PN	neurofibromatosis type 1- associated plexiform neurofibroma
nmDMD	nonsense mutation Duchenne muscular dystrophy
NSCLC	non-small cell lung cancer
oHCM	obstructive hypertrophic cardiomyopathy
OSA	obstructive sleep apnea
PAP	positive airway pressure
PKU	phenylketonuria
PsA	psoriatic arthritis
PsO	plaque psoriasis
PSVT	paroxysmal supraventricular tachycardia
PTLD	post-transplant lymphoproliferative disorder
PWS	Prader Willi syndrome
RDEB	recessive dystrophic epidermolysis bullosa
RSV	respiratory syncytial virus
SC	subcutaneous
siRNA	small interfering ribonucelic acid

 | Data is current as of 1/25/2025

CONTINUED

Glossary

Term	Definition
SOT	solid organ tranplant
SR-aGvHD	steroid-refractory acute graft vs. host disease
T2DM	type 2 diabetes mellitus
TGCT	tenosynovial giant cell tumor
TKI	tyrosine kinase inhibitor
UC	ulcerative colitis
uUTI	uncomplicated urinary tract infection
VMS	vasomotor symptoms
WAC	Wholesale Acquisition Cost

 | Data is current as of 1/25/2025

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