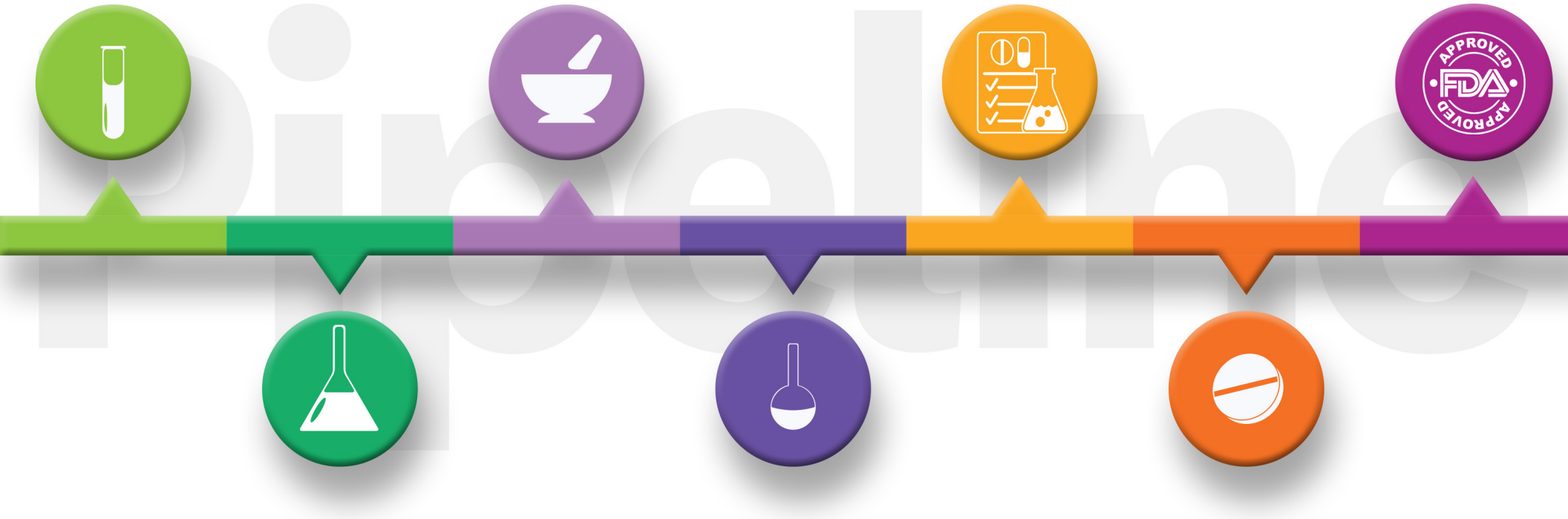

PIPELINE REPORT: JULY 2025

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.

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APPROVED: YEZTUGO® *(lenacapavir)* for pre-exposure prophylaxis of HIV-1 infection

APPROVED: EKTERLY® *(sebetralstat)* for treatment of acute attacks of hereditary angioedema

 | Data is current as of 7/22/2025

Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
DERMATOLOGY					
ZEVASKYN ™ *prademagene zamikeracel* epidermal sheet	Abeona Therapeutics	Recessive dystrophic epidermolysis bullosa (RDEB)	4/28/2025	**GENE THERAPY** · Approved for the treatment of wounds in adult and pediatric patients with RDEB. · ZEVASKYN is produced as autologous, COL7A1 gene-corrected epidermal sheets which are surgically applied 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. · Projected impact: new cost for a small population.	$3.1 million/ one-time treatment *The development of new RDEB lesions in different areas of the body would require additional courses of therapy.*
ENDOCRINOLOGY					
HARLIKU™ *nitisinone* oral tablet	Cycle Pharmaceuticals	Alkaptonuria	6/10/2025	· Approved for the reduction of urine homogentisic acid in adult patients with alkaptonuria. · Alkaptonuria is an ultra-rare genetic metabolic disorder that leads to osteoarthritis, ochronosis, and complications in the kidneys and heart. · HARLIKU is a low-dose alternative branded formulation of NITYR which is currently FDA-approved for the treatment of hereditary tyrosinemia type 1. · Projected impact: new cost for a small population.	$542,061.50/ 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					
ANDEMBRY® *garadacimab-gxii* SC injection	CSL Behring	Hereditary angioedema (HAE)	6/10/2025	• Approved for prophylaxis to prevent attacks of HAE in adult and pediatric patients ≥ 12 years of age. • Is the first HAE treatment to target activated Factor XIIa and offers once monthly SC dosing. • Will compete with multiple other available HAE prophylactic therapies including CINRYZE, HAEGARDA, ORLADEYO, and TAKHZYRO. • Projected impact: cost replacement of existing therapies.	$685,200/year
EKTERLY® *sebetralstat* oral tablet	KalVista Pharmaceuticals	HAE	7/3/2025	• Proposed for the on-demand treatment of HAE attacks in adult and pediatric patients ≥ 12 years of age. • EKTERLY is the first oral, on-demand therapy for people living with HAE. • Will compete with other HAE therapies used for on-demand treatment such as FIRAZYR (now generic), BERINERT, KALBITOR, and RUCONEST. • Projected impact: cost replacement of existing therapies.	$200,640/year (for 12 acute attacks per year)
INFECTIOUS DISEASES					
ENFLONSIA™ *clesrovimab-cfor* intramuscular injection	Merck	Respiratory syncytial virus (RSV) infection	6/9/2025	• Approved for the prevention of RSV lower respiratory tract disease in neonates and infants who are born during or entering their first RSV season. • One-time injection, same single dose regardless of weight. • ENFLONSIA will be covered under the Medicaid Vaccines for Children program. • BEYFORTUS and SYNAGIS are similarly FDA-approved. • Projected impact: cost replacement of existing therapies.	$556/one-time dose

 | Data is current as of 7/22/2025

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
YEZTUGO® *lenacapavir* oral tablet and SC injection	Gilead	HIV pre-exposure prophylaxis (PrEP)	6/18/2025	· Approved for PrEP to reduce the risk of sexually acquired HIV-1 in adults and adolescents (≥ 35kg) who are at risk for HIV-1 acquisition. · The YEZTUGO Prescribing Information includes a Boxed Warning re: the risk of drug resistance with use of YEZTUGO for HIV-1 PrEP in undiagnosed HIV-1 infection. Individuals must have a negative HIV-1 test prior to initiating YEZTUGO. · YEZTUGO is a twice-yearly alternative branded formulation of SUNLENCA which is currently FDA-approved for the treatment of HIV-1 infection in heavily treatment-experienced, multi-drug resistant adults. · TRUVADA (available generically), DESCOVY, and APRETUDE are also FDA-approved for HIV PrEP. · Projected impact: cost replacement of existing therapies.	$28,218/year
NEUROMUSCULAR DISEASES					
IMAAVY™ *nipocalimab-aahu* IV infusion	Johnson & Johnson	Generalized myasthenia gravis (gMG)	4/29/2025	· Approved for the treatment of generalized myasthenia gravis (gMG) in adult and pediatric patients ≥ 12 years of age who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive. · Will compete with SOLIRIS, ULTOMIRIS, VYVGART, VYVGART HYTRULO, and ZILBRYSQ in AChR antibody-positive patients, and with RYSTIGGO in AChR- or MuSK antibody-positive patients. · Projected impact: cost replacement of existing therapies.	$324,480/year
ONCOLOGY					
OPDIVO® *nivolumab* IV infusion	Bristol Myers Squibb	Hepatocellular carcinoma (HCC)	4/11/2025	• **New indication for an existing agent.** · Approved for combination use with YERVOY (ipilimumab) for the treatment of adult patients with unresectable or metastatic HCC, as a first-line treatment. · OPDIVO was previously approved in combination with YERVOY for the treatment of HCC in patients previously treated with sorafenib. · Projected impact: no significant incremental cost increase.	$202,470/year

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Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
penpulimab-kcqx IV infusion	Akeso, Inc.	Nasopharyngeal carcinoma (NPC)	4/23/2025	• Approved for use in combination with cisplatin or carboplatin and gemcitabine for the first-line treatment of adults with recurrent or metastatic non-keratinizing NPC; and as a single agent for the treatment of adults with metastatic non-keratinizing NPC with disease progression on or after platinum-based chemotherapy and at least one other prior line of therapy. • LOQTORZI is FDA-approved for similar NPC indications while KEYTRUDA is also used off-label. • Projected impact: cost replacement of existing therapies.	Pending launch
AVMAPKI™ FAKZYNJA™ CO-PACK *avutometinib + defactinib* oral capsule and oral tablet	Verastem Oncology	Ovarian cancer	5/8/2025	• Approved for the treatment of adult patients with KRAS-mutated recurrent low-grade serous ovarian cancer (LGSOC) who have received prior systemic therapy. • Projected impact: cost replacement of existing therapies.	$630,500/year
EMRELIS™ *telisotuzumab vedotin-tllv* IV infusion	AbbVie	Non-small cell lung cancer (NSCLC)	5/14/2025	• Approved for the treatment of adult patients with locally advanced or metastatic, non-squamous NSCLC with high c-Met protein overexpression who have received a prior systemic therapy. • Projected impact: cost replacement of existing therapies.	$581,568/ year per 80 kg person
IBTROZI™ *taletrectinib* oral capsule	Nuvation Bio	NSCLC	6/11/2025	• Approved for the treatment of adult patients with locally advanced or metastatic ROS1-positive NSCLC. • XALKORI, ROZLYTREK, and AUGTYRO are similarly FDA-approved. • Projected impact: cost replacement of existing therapies.	$353,856/year

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Recent Specialty Drug Approvals

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	FDA Approval Date	Comments	Cost (WAC) /Utilizer
ZUSDURI™ *mitomycin* intravesical instillation	UroGen	Bladder cancer	6/12/2025	• FDA-approved for the treatment of adult patients with recurrent low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). • ZUSDURI is a sustained release, hydrogel-based formulation designed to enable longer exposure of bladder tissue to mitomycin, providing a pharmacologic alternative to surgical treatment. • Projected impact: no significant incremental cost increase.	$129,000/six-dose course of therapy
KEYTRUDA® *pembrolizumab* IV infusion	Merck	Locally advanced head and neck squamous cell carcinoma (LA-HNSCC)	6/12/2025	• **New indication for an existing agent.** • Approved for the treatment of patients with resectable LA-HNSCC whose tumors express PD-L1 as a single agent as neoadjuvant treatment, continued as adjuvant treatment in combination with radiotherapy with or without cisplatin and then as a single agent. • KEYTRUDA is already approved as monotherapy and in combination regimens for appropriate patients with metastatic or with unresectable, recurrent HNSCC. • Projected impact: no significant incremental cost increase.	$212,318/year
ZEGFROVY® *sunvozertinib* oral tablets	Dizal	NSCLC	7/2/2025	• Approved for the treatment of adult patients with locally advanced or metastatic NSCLC with epidermal growth factor receptor (*EGFR*) exon 20 insertion mutations, 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. • 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
LYNOZYFIC™ *linvoseltamab-gcpt* IV infusion	Regeneron	Multiple myeloma (MM)	7/2/2025	• Approved for the treatment of adult patients with relapsed or refractory MM who have received at least four prior lines of therapy, including a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody. • The LYNOZYFIC Prescribing Information includes a Boxed Warning re: cytokine release syndrome and neurologic toxicity, including immune effector cell-associated neurotoxicity syndrome. • Multiple other agents, including CAR T-cell therapies such as ABECMA and CARVYKTI, are also FDA-approved for use as 5th-line or later therapy in relapsed or refractory MM. • Projected impact: cost replacement of existing therapies.	Year 1: $472,820 - $604,420 Year 2 and thereafter: $244,400 - $488,800/year *The price range reflects the differentiated cost based on response to therapy by Week 24 of treatment.*
RENAL DISEASES					
VANRAFIA® *atrasentan* oral tablet	Novartis	Immunoglobulin A nephropathy (IgAN)	4/2/2025	• Approved to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥ 1.5 g/g. • The VANRAFIA Prescribing Information includes a Boxed Warning re: embryofetal toxicity. • FABHALTA, FILSPARI, and TARPEYO are also FDA-approved for the treatment of patients with IgAN. • Projected impact: cost replacement of existing therapy.	$162,500/year

 | Data is current as of 7/22/2025

Specialty Products on Our Radar

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	12/26/2025
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						
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.	$200,000/ year	7/30/2025
UX111 $ *rebisufligene etisparvovec* IV infusion	Ultragenyx	Mucopolysaccharidosis type IIIA (MPS IIIA)	*SGSH* gene-directed gene therapy	**GENE THERAPY** • Proposed for the treatment of Sanfilippo syndrome type A (aka MPS IIIA). • MPS IIIA is a rare, fatal lysosomal storage disease with no approved treatment that primarily affects the central nervous system and is characterized by rapid neurodegeneration, with onset in early childhood. • In mid-July the FDA declined to approve UX111, citing manufacturing issues that Ultragenyx believes are readily addressable. The FDA's rejection letter did not note any review issues related to the clinical data package nor clinical inspections.	$3 million/ one-time treatment	Pending BLA re-submission

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

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Specialty Products on Our Radar

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
PTC743 $ *vatiquinone* oral therapy	PTC Therapeutics	Friedreich's ataxia (FA)	Glutathione biosynthesis enhancing agent	· Proposed for the treatment of children and adults living with FA. · If approved, vatiquinone would be the first FDA-approved therapy for children with FA; SKYCLARYS is FDA-approved for FA in patients ≥ 16 years of age.	$500,000/ year	8/19/2025
SL1009 $ *sodium dichloroacetate* oral solution	Saol Therapeutics	Pyruvate dehydrogenase complex deficiency (PDCD)	Pyruvate dehydrogenase kinase inhibitor	· PDCD is an ultra-rare mitochondrial disorder of carbohydrate oxidation that mostly affects the nervous system and skeletal muscle and leads to decreased ATP production and energy failure. · The U.S. prevalence of PDCD is estimated to be up to 2,000 people.	$750,000/ year	8/27/2025
MT1621 $ *doxecitine/doxribtimine* oral therapy	UCB Biosciences Inc.	Thymidine kinase 2 deficiency (TK2d)	Deoxynucleoside substrate enhancement therapy	· TK2d is an ultra-rare genetic disorder that results in mitochondrial dysfunction leading to inadequate energy production in cells. TK2d may present at all ages and causes progressive and severe muscle weakness, respiratory insufficiency, and is often fatal. · There are currently no FDA-approved therapies for TK2d.	$500,000/ year	August 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.**

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Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
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 three without treatment.	$750,000/ year	9/30/2025
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	3Q 2025
RGX-121 Ⓢ *clemidsogene lanparvovec* intracerebral injection	RegenXBio, Inc.	Mucopolysaccharidosis type II (MPS II)	Iduronate-2-sulfatase (I2S)-directed gene therapy	**GENE THERAPY** • Proposed for the treatment of Hunter syndrome (aka MPS II). • MPS II is estimated to occur in approximately 1 in 100,000 to 1 in 170,000 births. The current disease-modifying therapy for MPS II is enzyme replacement therapy with ELAPRASE administered intravenously; however, ELAPRASE does not adequately address the neurological manifestations of MPS II.	$3 million/ one-time treatment	11/9/2025

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

 | Data is current as of 7/22/2025

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Specialty Products on Our Radar

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ARO-APOC3 $ *plozasiran* SC injection	Arrowhead Pharmaceuticals	Familial chylomicronemia syndrome (FCS)	Apolipoprotein C-III (apoC-III)-targeting RNAi therapeutic	· Proposed as an adjunct to diet for reducing triglycerides in adult patients with FCS. · Will compete with TRYNGOLZA for the same indication.	$595,000/ year	11/18/2025
TRANSCON® CNP *navepegritide* SC injection	Ascendis Pharmaceuticals	Achondroplasia	C-type natriuretic peptide	· Proposed for the treatment of children with achondroplasia. · Dosed SC once weekly. · Would compete with VOXZOGO which is dosed SC once daily for the same indication.	$400,000/ year	11/30/2025
relacorilant $ oral therapy	Corcept Therapeutics	Hypercortisolism	Glucocorticoid II (GR-II) receptor antagonist	· Proposed for the treatment of patients with endogenous hypercortisolism (Cushing's syndrome). · Would compete with other agents which are FDA-approved or considered standard therapy for Cushing's disease or Cushing's syndrome such as KORLYM, ISTURISA, RECORLEV, *ketoconazole*, and SIGNIFOR.	$600,000/ year	12/30/2025
HEMATOLOGY						
IONIS-PKK-LRx $ *donidalorsen* SC injection	Ionis Pharmaceuticals	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
PRN1008 *rilzabrutinib* oral therapy	Principia Biopharma	Immune thrombocytopenic purpura (ITP)	Bruton's tyrosine kinase inhibitor	· Prednisone, immunoglobulins, RITUXAN, PROMACTA, NPLATE, TAVALISSE are existing treatment alternatives.	$200,000/ year	8/29/2025

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

 | Data is current as of 7/22/2025

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Specialty Products on Our Radar

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
OMS721 $ *narsoplimab* IV infusion	Omeros Corporation	Hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA)	Anti-MASP-2 monoclonal antibody	• Proposed for the treatment of high-risk HSCT-TMA in those who have persistent TMA despite modification of immunosuppressive therapy. • Administered once weekly for up to 8 weeks. • Persistent TMA is a life-threatening complication of HSCT with a reported mortality rate in high-risk patients of > 90%. There are no FDA-approved therapies, though some off-label usage occurs with agents such as SOLIRIS.	$500,000/ one-time course of therapy	9/1/2025
HEPATOLOGY						
GSK2330672 *linerixibat* oral tablet	GSK	Primary biliary cholangitis (PBC)	Inhibitor of the ileal bile acid transporter	• Proposed for the treatment of cholestatic pruritus in patients with PBC. • Ursodeoxycholic acid, IQIRVO, LIVDELZI, and OCALIVA are currently used for the treatment of PBC. • Cholestatic pruritus is a serious condition that can be debilitating, with patients experiencing sleep disturbance, fatigue, impaired quality of life.	$200,000/ year	3/24/2026

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

 | Data is current as of 7/22/2025

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IMMUNOLOGY						
etuvetidigene autotemcel Ⓢ IV infusion	Fondazione Telethon	Wiskott Aldrich syndrome (WAS)	*WAS* gene-directed gene therapy	**GENE THERAPY** · WAS is an X-linked primary immunodeficiency disorder caused by mutations in the WAS gene which encodes the WAS protein, a cytoskeletal regulator. People with WAS suffer from severe bleeding episodes which can be fatal, along with recurrent and relapsing infections, eczema, increased risk of developing autoimmune diseases and lymphomas. Without treatment, the median survival for *WAS* patients is 14 years of age. · Current treatment options include supportive therapies for managing and preventing clinical manifestations. The only potentially curative treatment is a hematopoietic stem cell transplant. · The estimated incidence of WAS is 1 to 10 cases per million males worldwide; the condition is rarer in females.	$4.5 million/ one-time treatment	4Q 2025
NEUROLOGY						
SAR442168 *tolebrutinib* oral therapy	Sanofi	Multiple sclerosis	Bruton's tyrosine kinase inhibitor	· Proposed for the treatment of adults with non-relapsing secondary progressive multiple sclerosis (nrSPMS).	$200,000/ year	9/28/2025

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

 | Data is current as of 7/22/2025

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NEUROMUSCULAR CONDITIONS						
CAP-1002 $ *deramiocel* IV infusion	Capricor Therapeutics/NS Pharma	DMD-associated cardiomyopathy	Anti-fibrotic, anti-inflammatory, angiogenic	**CELL THERAPY** • CAP-1002 is an allogeneic stromal cell therapy manufactured from donor heart tissue. • The initial target treatment population is those who are in the advanced stages of DMD (late ambulatory or non-ambulatory). • Administered as an IV infusion once every 3 months. • In mid-July the FDA declined to approve CAP-1002, citing that the BLA does not meet the statutory requirement for substantial evidence of effectiveness and the need for additional clinical data, along with some manufacturing issues. Capricor intends to submit additional clinical data from the Phase 3 HOPE-3 trial which are expected in 3Q 2025, in support of an eventual FDA approval.	$600,000/ year	Pending BLA re-submission
SRK-015 *apitegromab* IV infusion	Scholar Rock	Spinal muscular atrophy (SMA)	Myostatin activation inhibitor	• Proposed for the treatment of spinal muscular atrophy (SMA) in patients who are receiving SMN-targeted treatments. • Would be the first muscle-directed therapy approved for SMA. • When used as intended as adjunctive therapy, SRK-015 would increase the total cost of care over the existing cost for SMN-targeted treatments.	$400,000/ year	9/22/2025

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

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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. • A specific FDA approval date for TRANSLARNA has yet to be announced after the original estimated date of 4/30/25 has passed, with uncertainty around an ultimate FDA approval given the current delays at the FDA.	$750,000/ year	3Q 2025
BHV-4157 (S) *troriluzole* oral therapy	Biohaven	Spinocerebellar ataxia (SCA)	Glutamate modulator	• Troriluzole is a prodrug of riluzole which has been used off-label for the treatment of SCA. • SCA is a rare, debilitating neurodegenerative disorder that is estimated to affect approximately 22,000 people in the U.S. Standard of care treatment is supportive and there are currently no FDA-approved therapies.	$500,000/ year	4Q 2025
ZOLGENSMA (S) *onasemnogene abeparvovec-xioi* intrathecal injection	Novartis	SMA	*SMA* gene-directed gene therapy	**GENE THERAPY** • Proposed for the treatment of patients with SMA Type 2, up to 18 years of age. • This is an intrathecally delivered alternative formulation of the existing IV ZOLGENSMA product which is currently only FDA-approved for patients < 2 years of age. • The proposed indication represents an expansion of the potential market of ZOLGENSMA utilizers.	$2 million/ one-time treatment	December 2025

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

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ONCOLOGY						
REGN1979 *odronextamab* IV infusion	Regeneron	Follicular lymphoma (FL); diffuse large B-cell lymphoma (DLBCL)	CD20xCD3 bispecific antibody	• Proposed for the treatment of adult patients with relapsed/refractory (R/R) FL or R/R DLBCL, who have progressed after at least two prior systemic therapies.	$400,000/ year	7/30/2025
ONC201 *dordaviprone* oral therapy	Chimerix	Diffuse glioma	Mitochondrial caseinolytic protease P (ClpP)-targeting agent	• Proposed for the treatment of adult recurrent H3 K27M-mutant diffuse glioma.	$350,000/ year	8/18/2025
BI 1810631 *zongertinib* oral therapy	Boehringer Ingelheim	NSCLC	Tyrosine kinase inhibitor (TKI)	• Proposed for the treatment of adult patients with unresectable or metastatic NSCLC whose tumors have HER2 (ERBB2) mutations and who have received prior systemic therapy.	$300,000/ year	8/19/2025
PRGN-2012 Ⓢ *zopapogene imadenovec* SC injection	Precigen, Inc.	Recurrent respiratory papillomatosis (RRP)	Therapeutic vaccine	• PRGN-2012 is designed to elicit immune responses directed against cells infected with human papillomavirus (HPV) 6 or HPV 11. • Papillomas are benign, but in extremely rare cases can undergo cancerous transformation; additionally, although benign, papillomas can cause severe, life-threatening airway obstruction and respiratory complications. • The prevalence of RRP is estimated to be ~27,000 adults in the U.S.	$750,000/ treatment course	8/27/2025

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

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KEYTRUDA® SC *pembrolizumab + berahyaluronidase* SC infusion	Merck	NSCLC	PD-1 inhibitor	• **New SC formulation of an existing IV agent.** • Would compete with IV KEYTRUDA and SC/IV OPDIVO and other SC/IV checkpoint inhibitors (e.g., TECENTRIQ HYBREZA). • Administered every six weeks with a median injection time of two minutes.	$215,000/ year	9/23/2025
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	10/23/2025
BAY 2927088 *sevabertinib* oral therapy	Bayer	NSCLC	TKI	• Proposed for the treatment of adult patients with advanced NSCLC whose tumors have activating human epidermal growth factor receptors 2 (HER2) (ERBB2) mutations and who have received a prior systemic therapy. • Activating HER2 mutations are found in 2% to 4% of advanced NSCLC cases.	$300,000/ year	11/28/2025

 | Data is current as of 7/22/2025

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Specialty Products on Our Radar

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
ziftomenib oral therapy	Kura Oncology	Acute myeloid leukemia (AML)	Menin protein binder	• Proposed for the treatment of adult patients with relapsed/refractory acute myeloid leukemia (AML) with a nucleophosmin 1 (NP1) mutation. • In AML, NPM1 mutations are among the most common, representing ~30% of cases. While patients with NPM1 AML have high response rates to frontline therapy, relapse rates are high and survival outcomes are poor.	$300,000/ year	11/30/2025
RENAL DISEASES						
VIS649 *sibeprenlimab* SC injection	Otsuka	Immunoglobulin A nephropathy (IgAN)	Humanized IgG2 monoclonal antibody	• TARPEYO, FILSPARI, and VANRAFIA are also FDA-approved for IgAN. • Dosed as a self-administered SC injection every 4 weeks.	$200,000/ year	11/28/2025
RESPIRATORY DISEASES						
INS1007 *brensocatib* oral tablet	Insmed Inc.	Non-cystic fibrosis bronchiectasis (NCFB)	Dipeptidyl peptidase 1 (DPP1) inhibitor	• NCFB is a rare, chronic, inflammatory lung disease that has a marked impact on quality of life and predominantly affects females and older adults. • The current standard of care includes airway clearance, mucolytics, and antibiotics as needed.	$90,000/ year	8/12/2025
N115 *sodium pyruvate* intranasal spray	EmphyCorp Inc.	Idiopathic pulmonary fibrosis (IPF)	Anti-oxidative agent	• Proposed to reduce coughing in patients with IPF. • Current therapies approved for the treatment of IPF include oral ESBRIET and OFEV.	$150,000/ year	3Q 2025

 | Data is current as of 7/22/2025

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Specialty Products on Our Radar

Drug Name & Administration Method	Manufacturer(s)	Indication(s)	Mechanism(s) of Action	Comments	Anticipated Cost	Anticipated Approval Date
GSK3511294 *depemokimab* SC injection	GSK	Chronic rhinosinusitis with nasal polyps (CRSwNP); Asthma	IL-5 inhibitor	· Under FDA review for two proposed indications: for use as add-on maintenance treatment in adults with inadequately controlled CRSwNP and for use as add-on maintenance treatment of asthma in adult and pediatric patients ≥12 years of age with type 2 inflammation characterised by an eosinophilic phenotype on medium- to high-dose inhaled corticosteroids (ICS) plus another asthma controller. · Will compete with existing biologics already FDA-approved for CRSwNP and/or eosinophilic asthma such as CINQAIR, DUPIXENT, FASENRA,and NUCALA. · Ultra-long acting product with one dose administered every 6 months.	$50,000/ year	12/16/2025

 | Data is current as of 7/22/2025

Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
DERMATOLOGY						
STARJEMZA® *ustekinumab-hmny* SC injection	Bio-Thera Solutions	STELARA®	Plaque psoriasis; psoriatic arthritis; Crohn's disease; ulcerative colitis	FDA approval: 5/22/2025	Yes	· Is the eighth FDA-approved STELARA biosimilar, after IMULDOSA, OTULFI, PYZCHIVA, SELARSDI, STEQEYMA, WEZLANA, and YESINTEK. · The FDA granted interchangeable status for STARJEMZA to STELARA.
ENDOCRINOLOGY						
HLX14 *denosumab* SC injection	Shanghai Henlius Biotech, Inc.	PROLIA®, XGEVA®	Osteoporosis and fractures due to bone metastasis; skeletal-related bone events	BLA is under FDA review (BsUFA date: 8/30/2025)	No	· Would be a subsequent denosumab biosimilar, after multiple others.
AVT03 *denosumab* SC injection	Alvotech	PROLIA®, XGEVA®	Osteoporosis and fractures due to bone metastasis; skeletal-related bone events	BLA is under FDA review (BsUFA date: 12/15/2025)	No	· Would be a subsequent denosumab biosimilar, after multiple others.
denosumab biosimilar SC injection	Amneal	PROLIA®, XGEVA®	Osteoporosis and fractures due to bone metastasis; skeletal-related bone events	BLA is under FDA review (BsUFA date: 4Q 2025)	No	· Would be a subsequent denosumab biosimilar, after multiple others.
RGB-14 *denosumab* SC injection	Hikma	PROLIA®, XGEVA®	Osteoporosis and fractures due to bone metastasis; skeletal-related bone events	BLA is under FDA review (BsUFA date: 4Q 2025)	No	· Would be a subsequent denosumab biosimilar, after multiple others.
TVB-009P *denosumab* SC injection	Teva	PROLIA®, XGEVA®	Osteoporosis and fractures due to bone metastasis; skeletal-related bone events	BLA is under FDA review (BsUFA date: 2H 2025)	No	· Would be a subsequent denosumab biosimilar, after multiple others.

 | Data is current as of 7/22/2025

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Biosimilars

Drug Name & Administration Method	Manufacturer(s)	Biosimilar Reference Drug	Indication(s)	Status/Estimated Approval	Biosimilar Currently Launched?	Comments
IMMUNOLOGY						
AVT05 *golimumab* SC and IV injections	Alvotech	SIMPONI®	Rheumatoid arthritis (RA), psoriatic arthritis (PsA), polyarticular juvenile idiopathic arthritis (pJIA), ankylosing spondylitis (AS), ulcerative colitis (UC)	BLA is under FDA review (BsUFA date: 4Q 2025)	No	• Would be the first FDA-approved biosimilar to SIMPONI.
ONCOLOGY						
JOBEVNE™ *bevacizumab-nwgd* IV infusion	Biocon Biologics	AVASTIN®	Colorectal cancer, non-small cell lung cancer; glioblastoma; renal cell carcinoma; cervical cancer; ovarian, fallopian tube, or primary peritoneal cancer	FDA approval: 4/10/2025	Yes - ALYMSYS, MVASI, VEGZELMA, ZIRABEV	• Is the 6th biosimilar to AVASTIN, after ALYMSYS, AVZIVI, MVASI, VEGZELMA, and ZIRABEV.
OPHTHALMOLOGY						
CTP42 *aflibercept* intraocular injection	Celltrion	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 3Q 2025)	No	• Would be a subsequent EYLEA biosimilar, after AHZANTIVE, ENVEEZU, OPUVIZ, PAVBLU, and YESAFILI.
AVT06 *aflibercept* intraocular injection	Alvotech	EYLEA®	Wet AMD	BLA is under FDA review (BsUFA date: 4Q 2025)	No	• Would be a subsequent EYLEA biosimilar, after AHZANTIVE, ENVEEZU, OPUVIZ, PAVBLU, and YESAFILI.

Generic Specialty Agents

Recent Approvals			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
tolvaptan	JYNARQUE®	Lupin	5/12/2025
eltrombopag olamine	PROMACTA®	Hetero	5/13/2025
nilotinib hydrochloride	TASIGNA®	Apotex	5/27/2025
emtricitabine/rilpivirine hydrochloride/ tenofovir disoproxil fumarate	COMPLERA®	Mylan	5/27/2025
bosentan (dispersible tablet for oral suspension)	TRACLEER®	Natco Pharma	6/17/2025
Pipeline Agents*			
GENERIC NAME	**BRAND NAME**	**MANUFACTURER(S)**	**MARKET LAUNCH DATE**
stiripentol (oral capsule and powder)	DIACOMIT®	Unknown	8/20/2205
glycerol phenylbutyrate	RAVICTI®	Par/Endo	3Q 2025
rilpivirine hydrochloride	EDURANT®	Strides	10/22/2025
macitentan	OPSUMIT®	Alembic; Laurus Labs; MSN Laboratories; Mylan/Viatris; Seasons Biotechnology (Taizhou); Sun; Teva	4Q 2025
treprostinil	TYVASO®	Actavis/Teva	1/1/2026
pomalidomide	POMALYST®	Unknown	March 2026
nintedanib esylate	OFEV®	Accord; Glenmark	4/2/2026
melphalan hydrochloride	EVOMELA®	Aurobindo; Ligand Pharmaceuticals; Cydex Pharmaceuticals; Acrotech Biopharma	6/1/2026

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

 | Data is current as of 7/22/2025

Glossary

Term	Definition
ABR	annualized bleeding rate
ADC	antibody-drug conjugate
ALL	acute lymphoblastic leukemia
allo-HSCT	allogeneic hematopoietic stem cell transplantation
AMD	age-related macular degeneration
AML	acute myeloid leukemia
apo-CIII	apolipoprotein C-III
AS	ankylosing spondylitis
BCMA	B-cell maturation antigen
BLA	biologics license application
BsUFA	Biosimilar User Fee Act
BTC	biliary tract cancer
CAR T-cell	chimeric antigen receptor T-cell
CD	Crohn's disease
CDC	Centers for Disease Control and Prevention
CHE	chronic hand eczema
CKD	chronic kidney disease
CMS	Centers for Medicare & Medicaid Services
CNTF	ciliary neurotrophic factor
CRSwNP	chronic rhinosinusitis with nasal polyps
cSCC	cutaneous squamous cell carcinoma
CTX	cerebrotendinous xanthomatosis
DLBCL	diffuse large B-cell lymphoma
DMD	Duchenne muscular dystrophy
DPP1	dipeptidyl peptidase 1
EGFR	epidermal growth factor receptor
ERT	enzyme replacement therapy
FA	Friedreich's ataxia
FAK	focal adhesion kinase
FCS	familial chylomicronemia syndrome
FDA	Food and Drug Administration
FIX	factor IX
FL	follicular lymphoma
FVIII	factor VIII
GEJ	gastroesophageal junction
GLP-1	glucagon-like peptide-1
gMG	generalized myasthenia gravis
GR-II	glucocorticoid II
HAE	hereditary angioedema
HCC	hepatocellular carcinoma
HER	human epidermal growth factor receptor
HFpEF	heart failure with preserved ejection fraction
HR	hormone receptor
HSCT	hematopoietic stem cell transplantation
I2S	iduronate-2-sulfatase
ICS	inhaled corticosteroids
IgAN	IgA nephropathy
IPF	idiopathic pulmonary fibrosis
ITP	immune thrombocytopenic purpura
IV	intravenous
JAK	Janus Kinase
LA-HNSCC	locally advanced head and neck squamous cell carcinoma
LDH	lumbar disc herniation
LG-IR-NMIBC	low-grade intermediate-risk non-muscle invasive bladder cancer
MacTel	macular telangiectasia type 2
MASH	metabolic dysfunction–associated steatohepatitis
MDS	myelodysplastic syndrome
MEK 1/2	mitogen-activated protein kinase 1 and 2
MM	multiple myeloma
MPS II	mucopolysaccharidosis type II

 | Data is current as of 7/22/2025

CONTINUED

Glossary

Term	Definition
MPS IIIA	mucopolysaccharidosis type IIIA
NCFB	non-cystic fibrosis bronchiectasis
NF1	neurofibromatosis type 1
nmDMD	nonsense mutation Duchenne muscular dystrophy
NP1	nucleophosmin 1
nrSPMS	non-relapsing secondary progressive multiple sclerosis
NSCLC	non-small cell lung cancer
oHCM	obstructive hypertrophic cardiomyopathy
PAD	peripheral artery disease
PBC	primary biliary cholangitis
PDCD	pyruvate dehydrogenase complex deficiency
pJIA	polyarticular juvenile idiopathic arthritis
PKU	phenylketonuria
PN	plexiform neurofibroma
PrEP	pre-exposure prophylaxis
PsA	psoriatic arthritis
PsO	plaque psoriasis
PSVT	paroxysmal supraventricular tachycardia
PWS	Prader Willi syndrome
RA	rheumatoid arthritis

Term	Definition
RDEB	recessive dystrophic epidermolysis bullosa
RRP	recurrent respiratory papillomatosis
RSV	respiratory syncytial virus
SC	subcutaneous
SCA	spinocerebellar ataxia
siRNA	small interfering ribonucelic acid
sJIA	systemic juvenile idiopathic arthritis
SMA	spinal muscular atrophy
T2DM	type 2 diabetes mellitus
TGCT	tenosynovial giant cell tumor
TK2d	thymidine kinase 2 deficiency
TKI	tyrosine kinase inhibitor
UC	ulcerative colitis
UPCR	urine protein-to-creatinine ratio
uUTI	uncomplicated urinary tract infection
VMS	vasomotor symptoms
WAC	Wholesale Acquisition Cost
WAS	Wiskott Aldrich syndrome

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