carelon Rx

Drug and biologic pipeline update Q4 2025

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CarelonRx's quarterly Drug and biologic pipeline update

CarelonRx closely monitors the drug and biologic pipeline as part of our mission to improve health, lower the total cost of care across pharmacy and medical, and deliver an exceptional experience for our clients and members. Pipeline monitoring supports evidence-based, clinically appropriate use of drugs and therapies at the time of launch. In addition, future cost impact estimates provide valuable insights to guide health plan and client planning. Our Q4 2025 update highlights three agents in late-stage development: depemokimab for asthma and rhinosinusitis with nasal polyps, tinlarebant for Stargardt disease, and surabgene lomparvovec for wet age-related macular degeneration (wet AMD). An update on gene therapies and biosimilars is provided. An overview of the Food and Drug Administration (FDA) Commissioner's National Priority Voucher (CNPV) program is also featured. Additional topics include an update on the human immunodeficiency virus (HIV)-1 pre-exposure prophylaxis (PrEP) market and an overview of the pipeline for selective estrogen receptor degraders (SERDs) in breast cancer.

Emerging new therapies

Depemokimab

Condition:

Severe asthma with type 2 (T2) inflammation and CRSwNP are distinct but overlapping inflammatory diseases driven by T2 immune responses. Both are characterized by eosinophilic inflammation and IL-5–mediated pathways, which contribute to persistent symptoms, exacerbations, and reduced quality of life. Asthma affects over 25 million people in the United States (U.S.), with 5 to 10% experiencing severe disease despite high-intensity inhaled corticosteroid therapy and additional controllers. A large proportion of these individuals show evidence of T2 inflammation, often requiring systemic corticosteroids that increase the risk of long-term complications.

CRSwNP has an estimated prevalence of 1 to 4% in the US, with a higher incidence in individuals who also have asthma or allergic disease. Many individuals undergo repeated courses of systemic corticosteroids and sinus surgeries, yet relapse remains common. Importantly, CRSwNP and severe asthma frequently coexist, reflecting their shared inflammatory biology.

Role in treatment:

Individuals with severe asthma and CRSwNP continue to experience substantial unmet clinical need. Systemic corticosteroids remain a mainstay for disease control but carry risks when used repeatedly or long term, including osteoporosis, diabetes, infection, and cardiovascular complications. Surgical intervention is often pursued in CRSwNP, but recurrence is common, and many individuals require multiple procedures over their lifetime.

Biologic therapies targeting T2 inflammation have transformed the treatment landscape. For severe asthma, available options include Cinqair®, Dupixent®, Fasenra®, Nucala®, Tezspire®, and Xolair®. These agents require dosing intervals ranging from every 2 weeks to every 8 weeks.

Depemokimab, a long-acting anti–IL-5 monoclonal antibody, is being investigated as add-on maintenance therapy in adults and adolescents for both severe asthma with T2 inflammation (in addition to inhaled corticosteroids plus additional controller[s]) and CRSwNP (in addition to intranasal corticosteroids). In trials, depemokimab is administered once every 6 months, offering the potential for durable disease control with markedly reduced dosing frequency. This extended interval may improve adherence.

Additional indications for depemokimab are being studied, including eosinophilic granulomatosis with polyangiitis (EGPA), hypereosinophilic syndrome (HES), and chronic obstructive pulmonary disease (COPD) with T2 inflammation.

Efficacy:

The Biologics License Application (BLA) submitted to the Food and Drug Administration (FDA) for depemokimab is supported by data from the SWIFT-1 and SWIFT-2 phase 3 clinical trials in severe asthma and the ANCHOR-1 and ANCHOR-2 phase 3 clinical trials in CRSwNP.

In severe asthma, the SWIFT studies evaluated depemokimab in uncontrolled disease despite high-intensity standard therapy. Over 52 weeks, people receiving depemokimab experienced 0.46 and 0.56 exacerbations per year compared with 1.11 and 1.08 with placebo. Improvements in lung function and asthma control scores were also observed.

Product:
Depemokimab

Indication:
Severe asthma and chronic rhinosinusitis with nasal polyps (CRSwNP)

Estimated FDA approval:
December 2025

Therapeutic class:
Interleukin 5 (IL-5) antagonist

Route of administration:
Subcutaneous injection

FDA designations:
None

Manufacturer:
GlaxoSmithKline

In CRSwNP, depemokimab significantly reduced nasal polyp score by -0.7, and nasal obstruction score by -0.24 compared to placebo. Individuals also reported improvements in sense of smell and quality of life, and there was a decreased need for systemic corticosteroids and surgical intervention.

Emerging new therapies

Depemokimab

Safety:
In clinical trials, depemokimab was generally well tolerated, with a safety profile consistent with other biologics targeting IL-5.

Financial impact:
If approved, depemokimab would offer an additional therapeutic option for severe asthma and CRSwNP driven by T2 inflammation. Existing FDA-approved biologics for asthma cost an average of $30,000 to $40,000 per year, and a new product in this class would likely be priced similarly or at a modest premium. Depemokimab's twice-yearly dosing schedule may reduce administration burden and improve adherence. It is unclear if this will result in lowering indirect healthcare costs related to exacerbations, surgeries, and steroid-associated complications.

CarelonRx view:
Depemokimab will enter a competitive biologic class for severe asthma and CRSwNP. While likely to be viewed as clinically comparable in efficacy and safety to existing IL-5 inhibitors, its twice-yearly dosing offers a meaningful convenience advantage that may improve adherence and boost market share. The efficacy and safety of depemokimab for the additional indications studied is unknown at this time. If efficacy is confirmed in additional indications, such as EGPA, HES, or COPD, depemokimab's clinical value may increase.

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Indication:
Severe asthma and chronic rhinosinusitis with nasal polyps (CRSwNP)

Estimated FDA approval:
December 2025

Therapeutic class:
Interleukin 5 (IL-5) antagonist

Route of administration:
Subcutaneous injection

FDA designations:
None

Manufacturer:
GlaxoSmithKline

Tinlarebant

Condition:
Stargardt disease is a rare genetic eye condition that affects how your body uses vitamin A to make retinal cells. It occurs when the affected gene does not work and fatty materials build up on the macula, which is the part of the retina that is needed for central or sharp vision. Progressive loss of vision usually begins in adolescence and may lead to legal blindness. In addition to loss of central vision, this disease may cause sensitivity to light, color blindness, blind spots in the center of your vision, and occasional loss of peripheral vision. The speed of vision loss may vary among individuals but eventually becomes more pronounced, leading to challenges performing daily tasks, reading, and recognizing faces. Stargardt disease affects about 1 in 10,000 people in the U.S. and there is currently no treatment. Managing environmental factors such as exposure to UV light and smoking may help slow progression of vision loss. Aids including screen readers and magnifying glasses may be used to enhance remaining vision.

Role in treatment:
Tinlarebant would be the first FDA-approved treatment for Stargardt disease. By targeting retinol binding protein 4, tinlarebant works to reduce the accumulation of cytotoxic byproducts of vitamin A with the goal of intervening in the underlying pathophysiology of the disease.

Efficacy:
Tinlarebant has been evaluated in a phase 2 open-label trial that showed sustained lower atrophic lesion growth in adolescents on treatment compared to untreated individuals over 24 months. Initial results from the randomized, double-masked, placebo-controlled phase 3 DRAGON trial are expected later in 2025. This trial will be used for submission of a New Drug Application (NDA) to the FDA if results are positive.

Safety:
Tinlarebant was well-tolerated in the phase 2 trial throughout the 24-month treatment period. Safety will continue to be evaluated in the DRAGON trial, which will also last 24 months.

Financial impact:
If approved, tinlarebant will be the first treatment option for Stargardt disease. The cost is unknown at this time but is expected to be high based on pricing for other products approved to treat rare orphan diseases.

CarelonRx view:
In addition to being the first potential treatment for Stargardt disease, tinlarebant may provide the first oral option for another retinal disease, geographic atrophy (GA) in advanced dry age-related macular degeneration (dry AMD), which leads to vision loss in older adults. This additional approval may increase demand for the convenience of an oral option.

Product:
Tinlarebant

Indication:
Stargardt disease

Estimated FDA approval:
2026

Therapeutic class:
Retinol binding protein 4 antagonist

Route of administration:
Oral

FDA designations:
Breakthrough; Fast track; Orphan; Priority; Rare pediatric disease

Manufacturer:
Belite Bio

Surabgene lomparvovec

Condition:
Age-related macular degeneration (AMD) is a progressive eye disease and the leading cause of vision loss for those over the age of 65 in the United States. It is estimated that approximately 11 million Americans have AMD and less than 2 million of those are classified as having the neovascular (wet) subtype of AMD.

Diabetic retinopathy (DR) is an eye disease that can lead to vision loss for those with diabetes. It is estimated that approximately 8 million Americans have DR. Of those individuals, about 1 in 15 have diabetic macular edema (DME), another serious eye condition which can cause blurry vision due to leaking blood vessels in the eye.

AbbVie and Regenxbio plans to initially file for FDA-approval of surabgene lomparvovec (sura-vec) for the treatment of wet AMD, then later, plans to seek additional indications for DME and DR.

Role in treatment:
Guidelines recommend vascular endothelial growth factor (VEGF) inhibitors as first line therapy to treat and to stabilize leaky blood vessels in the eye causing both wet AMD and DME with vision loss. There are already several FDA-approved VEGF inhibitors on the market; however, each requires ongoing treatment with repetitive intravitreal injections, administered every 4 to 16 weeks.

If approved, sura-vec could provide a one-time subretinal gene therapy treatment that delivers a gene encoding for a monoclonal antibody fragment that, when translated, expresses the protein designed to inhibit VEGF. Sura-vec is not targeting an underlying genetic mutation or a genetic disease. It is believed to work by inhibiting the pathway by which the leaky blood vessels form and cause damage in wet AMD and DME.

Efficacy:
Trials are evaluating individuals 50 years of age and older, with wet AMD, who have demonstrated a meaningful response to FDA-approved VEGF inhibitor injections at study entry. Sura-vec, administered subretinally one-time, is being compared directly to an FDA-approved VEGF inhibitor administered intravitreally every 4 to 8 weeks. Phase 3 study results evaluating the change in vision from baseline to one year after treatment are expected in 2026.

Sura-vec is also advancing into late-stage trials evaluating another administration method using an in-office suprachoroidal microinjector, for both diabetic DR and wet AMD indications. If suprachoroidal injection is approved, this administration route would allow for a less invasive option presenting the potential for an in-office, outpatient type procedure for sura-vec.

Safety:
Early phase trial data demonstrated no sura-vec related serious adverse events. Sura-vec was well tolerated with its most common adverse events being mild retinal pigmentary changes and transient post-operative conjunctival hemorrhage.

Financial impact:
The price of sura-vec is unknown. However, it could be priced similarly to other gene therapies at $1M or more per one-time treatment.

CarelonRx view:
Like with other adeno-associated viral (AAV) vector delivered gene therapies, antibodies form after exposure to sura-vec that may limit redosing individuals who fail to respond to initial treatment.

Product:
Surabgene lomparvovec (sura-vec; also known as ABBV-RGX-314)

Indication:
Neovascular age-related macular degeneration (nAMD; also known as wet AMD)

Estimated FDA approval:
2026-2027

Therapeutic class:
Gene therapy

Route of administration:
Subretinal injection

FDA designations:
None

Manufacturer:
AbbVie and Regenxbio

While early trial data show the potential for sura-vec to decrease the frequency of intravitreal VEGF-inhibitor injections, it is unclear how long effects will last in individuals who respond to treatment. Phase 3 pivotal trial results could help answer lingering questions and help the industry better understand if there will be demand in the clinical community to use this new treatment with unknown durability in a disease state where the standard of care, repeat intravitreal injections, has well established efficacy and several FDA-approved treatment options.

Other significant product approvals

Other product approvals expected to reach the market in the next 12 months*

Drug or biologic manufacturer	Indication/route	Place in therapy	Estimated approval date	Impact on overall drug or medical spend
Troriluzole Biohaven	Spinocerebellar ataxia/ oral	**First in class:** would be first FDA-approved agent for this indication	Fourth quarter 2025	
Imlunestrant Eli Lilly	Hormone receptor (HR)+/ Human epidermal growth factor receptor 2 (HER2)- locally advanced or metastatic breast cancer/oral	**Addition to class:** for individuals previously treated with endocrine therapy; same class as Orserdu®	Fourth quarter 2025	
Lerodalcibep LIB Therapeutics	Dyslipidemia; Hypercholesterolemia/ subcutaneous (SC)	**Addition to class:** third generation proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor	12/12/2025	
Etripamil Milestone Pharmaceuticals	Supraventricular tachycardia/intranasal	**Addition to class:** rapid-response therapy that is self-administered	12/13/2025	
Zoliflodacin Innoviva	Gonorrhea/oral	**First in class:** novel mechanism of action; potential in resistant disease	12/15/2025	
Depemokimab GlaxoSmithKline	Asthma; Rhinosinusitis with nasal polyps/SC	**Addition to class:** potential advantage of every 6 month dosing	12/16/2025	
Reproxalap Aldeyra Therapeutics	Dry eye/ophthalmic	**First in class:** new mechanism of action for dry eye	12/16/2025	

In addition to treatments listed previously, these important drugs and biologics are scheduled to receive Food and Drug Administration (FDA) approval within the next 12 months.*

Orphan drug/rare disease; expected to be high cost, but with minimal impact to overall drug/medical spend due to low utilization

Potential to significantly increase overall drug/ medical spend

New entrant into current or future high-spend/ trending category

No significant impact to incremental spend due to replacement of existing competitors based on initial analysis

* As of September 16, 2025.

Other product approvals expected to reach the market in the next 12 months* (continued)

Drug or biologic manufacturer	Indication/route	Place in therapy	Estimated approval date	Impact on overall drug or medical spend
Aficamten Cytokinetics	Symptomatic obstructive hypertrophic cardiomyopathy (HCM)/oral	**Addition to class:** would compete with Camzyos®; quicker onset; potential for improved safety	12/26/2025	
Narsoplimab Omeros Corporation	Transplant-associated thrombotic microangiopathy/ intravenous (IV)	**First in class:** would be first FDA-approved treatment for this indication	12/26/2025	
Relacorilant Corcept	Cushing's syndrome/oral	**Addition to class:** next-generation competitive antagonist of the glucocorticoid II (GR-II) receptor	12/30/2025	
DNL310 (tividenofusp alfa) Denali Therapeutics	Mucopolysaccharidosis II/IV	**Addition to class:** would compete with Elaprase®; ability to cross blood brain barrier	01/05/2026	
Tabelecleucel Atara Biotherapeutics	Treatment of adult and pediatric individuals two years of age and older with Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)/IV	**First in class:** in individuals who have received at least one prior therapy	01/10/2026	
Sevabertinib Bayer	Advanced non-small cell lung cancer (NSCLC)/oral	**Addition to class:** for individuals whose tumors have activating human epidermal growth factor receptors 2 (HER2) (ERBB2) mutations and who have received a prior systemic therapy	01/28/2026	
Clemidsogene lanparvovec Regenxbio	Mucopolysaccharidosis II/IV	**First in class:** would be first gene therapy for this indication	02/08/2026	

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Other product approvals expected to reach the market in the next 12 months* (continued)

Drug or biologic manufacturer	Indication/route	Place in therapy	Estimated approval date	Impact on overall drug or medical spend
Bysanti (milsaperidone) Vanda Pharmaceuticals	Schizophrenia; Bipolar disorder/oral	**Addition to class:** active metabolite of iloperidone	02/21/2026	⊗
Etuvetidigene autotemcel Fondazione Telethon	Wiskott-Aldrich Syndrome (WAS)/IV	**First in class:** would be first FDA-approved treatment for this indication	03/11/2026	🔬
Linerixibat GlaxoSmithKline	Cholestatic pruritus in primary biliary cholangitis (PBC)/oral	**Addition to class:** would compete with Livdelzi®	03/24/2026	⊗
BBM-H901; Dalnacogene ponparvovec Belief Biomed	Hemophilia B/IV	**Addition to class:** gene therapy will compete with Hemgenix® in hemophilia B	04/12/2026	⊗
MK-8591A; islatravir/ doravirine Merck	Human immunodeficiency virus 1 (HIV)-1 treatment/oral	**First in class:** two-drug regimen will compete with three-drug regimens such as Biktarvy®	04/28/2026	⊗
Vepdegestrant Arvinas	HR+/HER2- locally advanced or metastatic breast cancer/oral	**Addition to class:** for individuals previously treated with endocrine therapy; same class as Orserdu	06/05/2026	⊗
Tabex (cytisinicline) Achieve Life Sciences	Smoking cessation/oral	**First in class:** naturally occurring plant alkyloid with binding affinity to the nicotinic acetylcholine receptor	06/26/2026	⊗

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🔬 Orphan drug/rare disease; expected to be high cost, but with minimal impact to overall drug/medical spend due to low utilization

📈 Potential to significantly increase overall drug/ medical spend

💲 New entrant into current or future high-spend/ trending category

⊗ No significant impact to incremental spend due to replacement of existing competitors based on initial analysis

* As of September 16, 2025.

Other product approvals expected to reach the market in the next 12 months* (continued)

Drug or biologic manufacturer	Indication/route	Place in therapy	Estimated approval date	Impact on overall drug or medical spend
Icotrokinra Johnson & Johnson	Plaque psoriasis, moderate-to-severe, ages 12 and older/oral	**First in class:** selective interleukin-23 receptor blocker	07/26/2026	⊗
CTX1301; Dexmethylphenidate Cingulate	Attention-deficit hyperactivity disorder (ADHD)/oral	**First in class:** once-daily, rapid onset	07/31/2026	⊗

In addition to treatments listed previously, these important drugs and biologics are scheduled to receive Food and Drug Administration (FDA) approval within the next 12 months.*

Orphan drug/rare disease; expected to be high cost, but with minimal impact to overall drug/medical spend due to low utilization

Potential to significantly increase overall drug/ medical spend

New entrant into current or future high-spend/ trending category

No significant impact to incremental spend due to replacement of existing competitors based on initial analysis

* As of September 16, 2025.

The Food and Drug Administration (FDA) requires all approved biologic products, including reference, biosimilar, and interchangeable products, be evaluated for safety and efficacy to determine whether the benefits outweigh any known potential risks.

Reference biologics undergo several phases of clinical studies to establish safety and effectiveness before they are FDA approved. Clinical trials begin with early, small-scale phase 1 studies and move toward late-stage, large-scale phase 3 studies. After the biologic has entered the market, post-marketing monitoring continues to assess the safety, efficacy, and clinical benefit in a larger population.

Biosimilar products are highly similar to their reference product in terms of structure and function, and lack clinically meaningful differences in terms of safety and efficacy. Biosimilar products may be approved for all or some of the reference product indications due to patent exclusivity.

Prescriptions for biosimilar products need to be written for the biosimilar by name. Biosimilar products that are granted interchangeability are allowed to be substituted for their reference biologic without the intervention of the prescriber. This is similar to how generic drugs may be substituted for brand-name drugs. Unlike reference biologics, biosimilar products are not required to submit evidence to establish safety and efficacy. However, a biosimilar manufacturer must submit clinical trial data that establishes biosimilarity with the reference product.

Biosimilar pipeline update

Currently seventy-three biosimilar products are FDA approved in the U.S. which represent nineteen unique reference biologic products. Recent approvals include Kirsty™ (insulin aspart-xjhz) in July 2025 and Bildyos® and Bilprevda® (denosumab-nxxp) in August 2025. Fifty-seven of the approved products have been launched.

Biosimilar products awaiting launch and/or approval*

Brand name	Brand manufacturer	Biosimilar name	Biosimilar manufacturer	FDA approval*
Actemra®	Roche; Chugai; Genentech	Avtozma®	Celltrion	1/24/2025
Avastin®	Genentech; Roche	Avzivi®	Bio-Thera Solutions; Sandoz	12/6/2023
		Jobevne	Biocon; Mylan; Viatris	4/9/2025
		FKB238	Centus Biotherapeutics; AstraZeneca; Fujifilm Kyowa Kirin	Pending
Enbrel®	Amgen; Immunex	Erelzi™	Sandoz	8/30/2016
		Eticovo™	Samsung Bioepis	4/25/2019
Eylea®	Regeneron	Ahzantive®	Formycon; Santo Holding; Bioeq; Klinge Pharma	6/28/2024
		Enzeevu™	Sandoz; Hexal	8/9/2024
		Opuviz™	Samsung Bioepis; Biogen	5/20/2024
		Yesafili™	Momenta; Mylan; Johnson & Johnson; Biocon; Viatris	5/20/2024
		AVT06	Alvotech; Teva; Alvogen	Pending
		CT-P42	Celltrion	Pending
Humalog®	Eli Lilly	GL-LIS	Gan & Lee; Sandoz	Pending
Humalog Pen				Pending
Humalog U-100 KwikPen				Pending
Lantus SoloStar®	Sanofi	GL-GLA	Gan & Lee; Sandoz	Pending

* As of September 16, 2025. Excludes biosimilars that are FDA approved and have launched.

Biosimilar products awaiting launch and/or approval* (continued)

Brand name	Brand manufacturer	Biosimilar name	Biosimilar manufacturer	FDA approval*
Lucentis®	Roche; Genentech	Lucamzi	Xbrane; Valorum Biologics; Stada	Pending
		LUBT010	Lupin	Pending
Neulasta®	Amgen	Lapelga	Apotex; Accord; Intas	Pending
Neupogen®	Amgen	Grastofil	Apotex; Accord; Intas	Pending
Novolog® (10 mL vial)	Novo Nordisk	Kirsty	Biocon	7/15/2025
Novolog FlexPen				
Novolog FlexTouch				
Novolog PenFill				
Novolog® (10 mL vial)	Novo Nordisk	Merilog™	Sanofi	2/14/2025
Novolog FlexPen				
Novolog FlexTouch				
Novolog PenFill				
Novolog® (10 mL vial)	Novo Nordisk	AMP-004	Amphastar	Pending
Novolog FlexPen				
Novolog FlexTouch				
Novolog PenFill				
Novolog (10 mL vial)	Novo Nordisk	GL-ASP	Gan & Lee; Sandoz	Pending
Novolog FlexPen				
Novolog FlexTouch				
Novolog PenFill				
Perjeta®	Genentech; Roche	HLX11	Henlius; Organon	Pending

* As of September 16, 2025. Excludes biosimilars that are FDA approved and have launched.

Biosimilar products awaiting launch and/or approval* (continued)

Brand name	Brand manufacturer	Biosimilar name	Biosimilar manufacturer	FDA approval*
Prolia®/Xgeva®	Amgen	Bildyos/Bilprevda	Henlius; Organon	9/2/2025
		Ospomyv™/Xbryk™	Samsung Bioepis; Samsung Biologics	2/13/2025
		AVT03	Alvotech; Dr. Reddy's; Alvogen	Pending
		Bmab 1000	Biocon	Pending
		ENZ215	Enzene; Alkem Labs	Pending
		INTP23	Intas; Accord	Pending
		MB09	mAbxience; Insud Pharma; Fresenius Kabi; Amneal	Pending
		RGB-14	Gedeon Richter; Hikma	Pending
		TVB-009P	Teva	Pending
Simponi®/Simponi Aria	Johnson & Johnson	AVT05	Alvotech; Teva; Alvogen	Pending
		BAT2506	Bio-Thera Solutions; Accord; Intas	Pending
Stelara®	Johnson & Johnson	Starjemza	Bio-Thera Solutions; Hikma	5/22/2025
Tysabri® IV	Biogen; Royalty Pharma	Tyruko®	Polpharma; Sandoz	8/24/2023
Xolair®	Roche; Genentech; Novartis	Omlyclo	Celltrion	3/7/2025

* As of September 16, 2025. Excludes biosimilars that are FDA approved and have launched.

Gene therapies in the pipeline

Gene therapy introduces or edits genetic material to treat disease. We group these drugs into (1) single-use gene therapies that aim to cure inherited conditions and (2) gene-based therapeutics that require repeat dosing. Because prices are disclosed only after FDA approval, forecasting remains difficult, but most forthcoming gene therapies are likely to launch in the $2 - 4 million range, consistent with recent approvals.

The following gene therapies and gene-based therapeutics are scheduled to receive an FDA decision in the next 12 months, or we expect they could file a biologics license application (BLA) with the FDA before the end of 2027.

Gene and gene-based therapies with submitted applications for potential FDA-approval in 2025/2026*

Gene therapy/ gene-based therapy	Indication/route and frequency	Place in therapy	Estimated approval date
Zolgensma® (onasemnogene abeparvovec) Novartis	Spinal muscular atrophy (SMA) Type 2/single intrathecal infusion	Potential expanded indication for Zolgensma to include children 2 to < 18 years of age with SMA Type 2; will compete with Spinraza® and Evrysdi®. Uses viral vector (adeno-associated virus).	12/30/2025 (filed)
Clemidsogene lanparvovec Regenxbio	Mucopolysaccharidosis II (MPS II; Hunter syndrome)/single intracisternal or intracerebroventricular injection	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	02/08/2026 (filed)
Etuvetidigene autotemcel Fondazione	Wiskott-Aldrich Syndrome/single intravenous (IV) infusion	First gene therapy for this indication. Uses viral vector (lentivirus).	03/11/2026 (filed)
Dalnacogene ponparvovec (BBM-H901) Belief BioMed	Hemophilia B/single IV infusion	Third gene therapy for this indication; will compete with Hemgenix. Uses viral vector (adeno-associated virus).	04/12/2026
Pariglasgene brecaparvovec Ultragenyx	Glycogen storage disease type Ia/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026 (initiated rolling BLA)
Rebisufligene etisparvovec Abeona	Mucopolysaccharidosis IIIA (Sanfilippo Type A)/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026 (FDA-denied; plans to refile)
Sonpiretigene isteparvovec Nanoscope	Retinitis Pigmentosa (RP)/single intravitreal injection	First mutation-agnostic gene therapy for RP. Uses viral vector (adeno-associated virus).	2026 (initiated rolling BLA)

* As of September 16, 2025

Gene and gene-based therapies of significant interest with potential FDA-submissions in 2026/2027*

Gene therapy/ gene-based therapy	Indication/route	Place in therapy	Estimated approval date
AAV-AIPL1 MieraGTx	Leber Congenital Amaurosis 4 (LCA4)/single subretinal injection	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026
AMT-130 uniQure	Huntington's disease/stereotaxic surgery with single infusion into the brain	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026
Bidridistrogene xeboparvovec Sarepta	Limb-girdle muscular dystrophy (LGMD) Subtype 2E/R4/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026 (trials on hold)
Botaretigene sparoparvovec Johnson & Johnson	X-linked retinitis pigmentosa (XLRP)/single subretinal injection	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026
Cretostimogene grenadenorepvec Novartis	Bacillus Calmette-Guérin (BCG) unresponsive, non-muscle invasive bladder cancer (NMIBC)/multiple intravesical doses	Second gene-based therapeutic; would compete with Adstiladrin®. Uses viral vector (adeno-associated virus).	2026
Giroctocogene fitelparvovec Sangamo	Hemophilia A/single IV infusion	Second gene therapy for hemophilia A; will compete with FVIII products, Hemlibra, and Roctavian. Uses viral vector (adeno-associated virus).	2026
Isaralgagene civaparvovec Sangamo	Fabry disease/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026
KB803 (beremagene geperpavec) Krystal Biotech	Dystrophic epidermolysis bullosa (DEB)/multiple ophthalmic doses	First ophthalmic formulation of Vyjuvek™ to treat ocular complications secondary to DEB. Uses viral vector (herpes simplex virus).	2026
Rivunatpagene miziparvovec Ultragenyx	Wilson disease/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026
SGT-003 Solid Biosciences	Duchenne muscular dystrophy (DMD)/single IV infusion	Competing to be second gene therapy for DMD; will compete with Elevidys. Uses viral vector (adeno-associated virus).	2026
Avalotcagene ontaparvovec Ultragenyx	Ornithine transcarbamylase (OTC) deficiency/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026–2027
NTLA-2002 Intellia	Hereditary angioedema (HAE)/ single IV infusion	First gene therapy for this indication. Uses gene editing, delivered by lipid nanoparticles.	2026–2027

* As of September 16, 2025

Gene and gene-based therapies of significant interest with potential FDA-submissions in 2026/2027*

Gene therapy/ gene-based therapy	Indication/route	Place in therapy	Estimated approval date
OCU400 Ocugen	Retinitis pigmentosa (RP)/single subretinal injection	Potential to be first gene therapy for RP associated with *RHO* mutations; may also get approval for people with any other RP associated mutation with a clinical phenotype of RP. Uses viral vector (adeno-associated virus).	2026–2027
RGX-202 Regenxbio	Duchenne muscular dystrophy (DMD)/single IV infusion	Second gene therapy for DMD; will compete with Elevidys. Uses viral vector (adeno-associated virus).	2026–2027
RP-A501 Rocket	Danon disease/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026–2027
Surabgene lomparvovec Regenxbio	Neovascular age-related macular degeneration (wet AMD)/single subretinal and/or suprachoroidal injection	First gene therapy for this indication; will compete with treatments requiring multiple intravitreal injections such as Eylea® and Vabysmo®. Uses viral vector (adeno-associated virus).	2026–2027
TG-C Kolon TissueGene	Osteoarthritis of the knee/ multiple intraarticular injections	First gene-based therapeutic for this indication; potential to compete with intraarticular steroid injections and knee replacement surgery. Uses viral vector (retrovirus).	2026–2027
AAV-AQP1 MeiraGTx Holdings	Radiation-Induced Xerostomia/ single intraparotid injection	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027
AAV-GAD MeiraGTx	Parkinson's disease/single surgical infusion into the brain	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027
Aglatimagene besadenovec Candel	Intermediate-to-high-risk localized prostate cancer/ multiple intratumoral injections	First localized gene-based viral immunotherapy for this indication; used in combination with an oral anti-herpes drug, such as valacyclovir, to destroy cancer cells. Uses viral vector (adeno-associated virus).	2027
BBP-812 Aspa	Canavan disease/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027
Dabocemagene autoficel Castle Creek Biosciences	Dystrophic epidermolysis bullosa (DEB)/multiple intradermal injections	Third localized gene-based wound therapeutic for this indication; will compete with Vyjuvek and Zevaskyn™. Uses viral vector (lentivirus).	2027
Detalimogene voraplasmid enGene	BCG unresponsive, NMIBC/ multiple intravesical instillations	Third gene-based therapeutic for NMIBC; will compete with Adstiladrin and cretostimogene grenadenorepvec, if approved. Uses viral vector (adeno-associated virus).	2027

* As of September 16, 2025

Gene and gene-based therapies of significant interest with potential FDA-submissions in 2026/2027*

Gene therapy/ gene-based therapy	Indication/route	Place in therapy	Estimated approval date
Elevidys (delandistrogene moxeparvovec-rokl) Sarepta	Duchenne muscular dystrophy (DMD)/single IV infusion	Potential to expand approval to include individuals 4 years of age and younger with DMD. Uses viral vector (adeno-associated virus).	2027
Engensis; donaperminogene seltoplasmid Helixmith	Diabetic peripheral neuropathy and Diabetic foot and other ulcers/multiple intramuscular injections	First gene-based therapeutic for these indications. Uses non-viral vector (plasmid deoxyribonucleic acid (DNA)).	2027
IMNN-001 Imunon	Newly diagnosed advanced ovarian, fallopian tube, or peritoneal cancers/ multiple intraperitoneal infusions	First gene-based oncolytic immunotherapy for these indications; used in combination with chemotherapy. Uses a DNA plasmid vector enclosed in a nanoparticle delivery system.	2027
Laruparetigene zovaparvovec Beacon	X-linked retinitis pigmentosa (XLRP)/single subretinal injection	Second gene therapy for this indication; potential to compete with botaretigene sparoparvovec, if its approved. Uses non-viral vector (Dually Derivatized Oligochitosan® (DDX) platform).	2027
LX2006 Lexeo	Friedreich's Ataxia Cardiomyopathy/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027
Tavokinogene telseplasmid (TAVO) OncoSec Medical	Metastatic melanoma/multiple intratumoral injections	Addition to class; gene-based oncolytic immunotherapy; used in combination with Keytruda®; uses non-viral vector (plasmid DNA).	2027
OCU410ST Ocugen	Stargardt disease/single subretinal injection	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027–2028
OTL-203 Orchard	Mucopolysaccharidosis I (MPS I; Hurler Syndrome)/single IV infusion	First gene therapy for this indication. Uses viral vector (lentivirus).	2027–2028
TSHA-102 Taysha Gene Therapies	Rett Syndrome/single intrathecal infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027–2028

* As of September 16, 2025

Food and Drug Administration (FDA) National Priority Voucher program

The FDA recently announced the Commissioner's National Priority Voucher (CNPV) program. A voucher may be redeemed by drug developers in order to participate in a priority program that shortens the drug review time from approximately 10-12 months to 1-2 months.

This program will use a new process that gathers various physicians and scientists for a team-based one-day review rather than an application being sent to numerous FDA offices. The goal of this approach is to reduce inefficiencies and make timely decisions while upholding rigorous safety and efficacy standards. Pharmaceutical companies will be able to submit the majority of the drug application prior to completion of a clinical trial and have frequent communications with the FDA. If a drug meets requirements the agency may also grant an accelerated approval.

The FDA is planning to provide a limited number of vouchers in the first year of the program to companies aligned with certain U.S. national priorities which include addressing a U.S. health crisis, providing innovative cures, and increasing domestic drug manufacturing. The vouchers may be directed to a specific investigational product or be an undesignated voucher that can be used at the company's discretion.

The FDA hopes to create more efficiency while better meeting growing public health needs by providing significant new treatments.

Market trends

Human immunodeficiency virus (HIV) pre-exposure prophylaxis (PrEP) treatment landscape and pipeline

HIV pre-exposure prophylaxis (PrEP) is the prevention of HIV infection using medications. PrEP is for people without HIV who may be exposed to HIV through sex or injection drug use. PrEP is even more effective when it is combined with condoms and other prevention tools.

Daily oral PrEP therapies Truvada® (emtricitabine and tenofovir disoproxil fumarate or FTC/TDF; available as a generic) and Descovy® (emtricitabine and tenofovir alafenamide or FTC/TAF) are the mainstay for many individuals; however, longer-acting injectable options have gained interest. Truvada and Descovy are also FDA-approved to treat HIV infection, in combination with other antiretroviral agents.

Yeztugo® (lenacapavir) subcutaneous injection, the newest FDA-approved drug for HIV PrEP, is administered less frequently (twice yearly in the abdomen or thigh) than other agents for HIV PrEP. Yeztugo creates a potential for shift in individual preference, especially from those currently using injectable Apretude (cabotegravir) intramuscular injection (administered every two months in the muscle of the buttock). Disadvantages of Yeztugo include injection site reactions, which may form a nodule at the injection site about the size of a grape and last up to nine months. Both injectable Apretude and Yeztugo require administration by a healthcare professional and require initial oral dosing prior to injections.

The Centers for Disease Control and Prevention (CDC) HIV PrEP guidelines have not been updated since the approval of Yeztugo. Currently recommended medications include Truvada, Descovy and Apretude. No medication is broadly preferred over another. Considerations for HIV PrEP medications include kidney function, adherence and tolerance for injection site reactions.

On-demand (or 2-1-1) dosing of oral PrEP is not approved by the FDA and not recommended by the CDC. However, CDC guidance states on-demand dosing may be prescribed off-label for certain individuals. When using 2-1-1 dosing, the individual takes oral doses based on when they plan to have sex: two pills 24 hours before sex, one pill 24 hours after the first two-pill dose, and one pill 48 hours after the first two-pill dose.

Phase 3 studies are underway for Apretude's three-times-per-year intramuscular injection. Yeztugo once-yearly intramuscular injection is also being studied.[1] A once-monthly oral tablet (MK-8527) is in clinical trials versus generic Truvada with results expected in 2027.

The table below compares current options for HIV PrEP. Reported efficacy results are from controlled clinical trials. Real-world effectiveness may be decreased by factors including access, adherence and treatment stigma.

Efficacy comparison of FDA-approved medications for HIV PrEP

	Truvada or generic	Descovy	Apretude	Yeztugo
Efficacy (incident HIV infection rate)	Truvada 1-3% Placebo 4-5%	Descovy 0.3% Truvada 0.6%	Apretude 0.3-0.6% Truvada 1.7-2.2%	Yeztugo 0-0.0009% Truvada 0.8-1.5% Descovy 2%

Selective estrogen receptor degraders (SERDs) for breast cancer treatment landscape and pipeline

While the incidence of breast cancer has remained steady over the past two decades, advances in therapy have created a substantial decline in death rate. One reason for this progress is the increasing use of precision medicine, which matches treatments to the biology of an individual's cancer.

Through biomarker testing of tumor tissue, clinicians can determine if cancer cells carry increased levels of estrogen or progesterone receptors (ER or PR), or human epidermal growth factor type 2 receptors (HER2). These results not only guide which therapies are most likely to work, but also spare individuals from treatments unlikely to be effective. It is estimated that 67-80% of breast cancers in women are ER-positive (ER+), making therapies that target this pathway highly relevant.

Among those with ER+, HER2-negative (HER2-) advanced breast cancer, new treatment avenues are opening. A promising area of research is the development of SERDs. In 2023, the FDA approved the first oral SERD, Orserdu™ (elacestrant), for second-line or greater postmenopausal women and adult men with ER+, HER2-, estrogen receptor 1 (ESR1)-mutated advanced or metastatic disease. Multiple pharmaceutical companies are now advancing their own SERDs through clinical pipelines. These agents act by binding to the estrogen receptor and marking it for destruction, effectively blocking estrogen-driven tumor growth.

This matters because, although hormone therapy has long been a cornerstone of breast cancer care, current options have important limitations. Injectable therapies such as Faslodex® (fulvestrant; available as a generic) administered intramuscularly into the buttocks once monthly, can be burdensome, and selective estrogen receptor modulators (SERMs) like oral Soltamox® (tamoxifen; available as a generic) are prone to resistance. Oral SERDs may not only offer greater convenience but also overcome resistance with ESR1 mutations, a critical step forward for those with limited options.

Innovation, however, comes with trade-offs. While many established endocrine therapies are now available as low-cost generics, newly approved SERDs are expected to launch at significantly higher prices. For diagnosed individuals and providers, this raises important questions about value: how will the potential for improved outcomes and convenience balance against affordability and access?

Because a large percentage of breast cancers are ER+, advances in SERDs could affect care for the majority of people diagnosed with this disease. The table below summarizes approved and late-stage SERDs. While most are initially positioned in those who have progressed after prior endocrine therapy, several programs are exploring earlier use in the treatment pathway—a shift that could expand access and potentially change standards of care.

Approved and pipeline SERDs for ER+/HER2- advanced breast cancer

SERD	Company	Status	Anticipated Place in Therapy
Orserdu (elacestrant)	Menarini	FDA approved; seeking additional indications	ER+/HER2-, ESR1-mutated, advanced breast cancer following progression, In combination with everolimus Node positive, ER+/HER2-, early breast cancer with high risk of recurrence
Camizestrant (SERD; complete ER antagonist)	AstraZeneca	Filed for US approval	ER+/HER2- breast cancer, with or without Verzenio® (abemaciclib) Extended adjuvant treatment of HR+/HER2- breast cancer First line treatment of HR+/HER2- ESR1-mutated breast cancer, in combination with a cyclin dependent kinase 4/6 inhibitor (CDK4/6i) First line treatment of HR+/HER2- breast cancer, in combination with Ibrance® (palbociclib)
Imlunestrant	Eli Lilly	Filed for US approval	Second line treatment of ER+/HER2- metastatic breast cancer alone or in combination with Verzenio
Vepdegestrant	Pfizer/ Arvinas	Filed for US approval	ER+/HER2- advanced or metastatic breast cancer following progression on (CDK4/6i) and endocrine therapy
Palazestrant	Olema	Anticipated filing in 2026	Second or thirrd line treatment of ER+/HER2- metastatic breast cancer
Giredestrant	Roche	Anticipated filing in 2026	First line treatment of ER+/HER2- metastatic breast cancer, in combination with Ibrance

References

1. AVAC. *Years Ahead in HIV Prevention Research: Time to Market* (accessed August 2025): avac.org

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