carelon Rx

Drug and biologic pipeline update Q1 2026

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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 Q1 2026 update highlights three agents in late-stage development:

- Icotrokinra for psoriasis
- Lonvoguran ziclumeran (lonvo-z) for hereditary angioedema
- Pelacarsen for dyslipidemia with elevated lipoprotein(a)

You will also find an overview of other products that are expected to reach the market in the next 12 months and an update on gene therapies and biosimilars.

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Emerging new therapies

Icotrokinra

Condition:

Chronic plaque psoriasis is a long-lasting skin disease affecting about 8 million individuals in the United States. Psoriasis is likely to be underdiagnosed among African American and Black people, as well as other people of color due to differences in clinical presentation.

Plaque psoriasis causes red scaly patches on the skin and is linked to an overactive immune response. Psoriasis involving high-impact sites (e.g., the scalp, genitals, and hands and feet) can be difficult to treat and significantly diminish health-related quality of life. Psoriasis is often associated with other health conditions, including arthritis, depression, and heart disease. Treatment of chronic plaque psoriasis is generally pursued because of the negative effects psoriasis can have on quality of life. Current treatments help many people, though some do not respond well or treatments lose effectiveness over time.

Role in treatment:

Although multiple subcutaneous (SC) or intravenous (IV) biologic therapies targeting IL-17, IL-23, and tumor necrosis factor (TNF) pathways are available for chronic plaque psoriasis, oral options that are effective and well-tolerated are more limited. Oral options include acitretin, cyclosporine, methotrexate, Otezla® (apremilast, a phosphodiesterase 4 inhibitor), and Sotyktu® (deucravacitinib, a tyrosine kinase 2 inhibitor). Dosing frequency for oral systemic psoriasis therapy varies from once-daily to once-weekly. Topical options are also available for mild-to-moderate psoriasis.

Icotrokinra is designed to block the IL-23 pathway, which plays a role in skin inflammation.

Other agents for psoriasis that target IL-23 are given via SC or IV injection: Ilyuma®, Skyrizi®, Tremfya®, and IL-12/23 inhibitor ustekinumab (Stelara® and biosimilars). Icotrokinra is a once-daily oral tablet and is being studied for ongoing treatment of adults and adolescents with psoriasis who need systemic therapy or phototherapy.

Other agents of interest in phase 3 development include: zasocitinib and envudeucitinib, oral tyrosine kinase 2 inhibitors for psoriasis and psoriatic arthritis, piclidenoson, an oral first in class adenosine A3 receptor agonist for psoriasis, Sotyktu for psoriatic arthritis, and Ilyuma for nail psoriasis.

Efficacy:

Data in adults and adolescents from the phase 3 clinical trials ICONIC-LEAD, ICONIC-ADVANCE, and ICONIC-TOTAL supported the FDA submission for icotrokinra.

Icotrokinra was superior to both placebo (primary outcome) and Sotyktu (secondary outcome) in the ICONIC-ADVANCE studies in adults with moderate-to-severe plaque psoriasis. Icotrokinra improved severity of redness, scaling, and induration (how raised or hardened a plaque is) as measured by Psoriasis Area and Severity Index (PASI) and more individuals achieved clear or almost clear skin with icotrokinra as measured by Investigator's Global Assessment (IGA).

In ICONIC-TOTAL, icotrokinra improved skin clearance overall and in the scalp and genital areas of adults and adolescents at least 12 years old with plaque psoriasis involving high-impact areas (scalp, genital, hand, or foot). The difference in psoriasis clearance in the hands and feet was not statistically significant between icotrokinra and placebo. Other biologics have demonstrated efficacy in high impact areas including hands and feet.

Response rates were consistent among both biologic-naïve and biologic-experienced individuals, suggesting potential use as either a first-line or subsequent-line biologic therapy. Larger studies are ongoing to confirm these results and evaluate long-term outcomes over three years.

Primary results from a head-to-head plaque psoriasis study comparing icotrokinra to ustekinumab are expected in first quarter of 2026. This is the first direct comparison of an oral vs. injectable psoriasis therapy.

Product:
Icotrokinra

Indication:
Moderate-to-severe chronic plaque psoriasis

Estimated FDA approval:
July 2026

Therapeutic class:
Interleukin-23 (IL-23) antagonist

Route of administration:
Oral

FDA designations:
None

Manufacturer:
Johnson & Johnson

Emerging new therapies

Icotrokinra

Safety:
Icotrokinra has been generally well-tolerated in studies with rates of adverse events similar to placebo. The most common side effects were cold-like symptoms. No major safety concerns have been identified in clinical studies. Adverse event rates at week 24 were lower with icotrokinra than with Sotyktu in ICONIC-ADVANCE, including gastrointestinal events and infections.

Financial impact:
If approved, icotrokinra is expected to be priced similarly to existing biologic psoriasis treatments which cost approximately $100,000 to $200,000 per year. Its overall clinical value will depend on how well it works, how long benefits last, and whether it helps reduce disease flares and healthcare costs over time.

Potential future expanded uses in psoriatic arthritis, ulcerative colitis, and Crohn's disease may increase overall impact of icotrokinra.

CarelonRx view:
Icotrokinra will enter a highly competitive psoriasis biologic market dominated by IL-17 and IL-23 inhibitors with established efficacy and provider familiarity. If long-term studies confirm durable responses and a favorable safety profile, icotrokinra may find a targeted role among individuals needing alternative options. Its once-daily oral dosing may offer an alternative to injectable options.

Product:
Icotrokinra

Indication:
Moderate-to-severe chronic plaque psoriasis

Estimated FDA approval:
July 2026

Therapeutic class:
Interleukin-23 (IL-23) antagonist

Route of administration:
Oral

FDA designations:
None

Manufacturer:
Johnson & Johnson

Emerging new therapies

Lonvoguran ziclumeran

Condition:
Hereditary angioedema (HAE) is a rare disorder that leads to recurrent episodes, or attacks, of severe swelling in the skin, upper respiratory tract, and gastrointestinal tract. HAE affects approximately 6,500 people in the United States. The two most common types of HAE are caused by a deficiency or dysfunction in C1 esterase inhibitor (C1-INH), which helps regulate the plasma bradykinin cascade. Attacks are a result of an overproduction of bradykinin with symptoms affecting daily activities and in severe cases resulting in death. While frequency and duration vary, without treatment, episodes can occur every 1 to 2 weeks and resolve in a few days' time.

Role in treatment:
There is no cure for HAE. Current treatments can be grouped into two broad categories: on-demand treatments, which treat attacks when they occur, and prophylactic treatments, which aim to prevent future attacks. All individuals with HAE should have access to on-demand therapy to treat acute attacks. Individuals who experience frequent or severe HAE attacks may also be considered for long-term prophylaxis (LTP). Several Food and Drug Administration (FDA)-approved HAE LTP treatments are already on the market; however, each requires ongoing treatment and most are given via injection, administered every 2 to 4 weeks.

Lonvoguran ziclumeran (lonvo-z) is an in-vivo clustered regularly interspaced short palindromic repeats (CRISPR)-based gene editing therapy, delivered via non-viral lipid nanoparticles (LNP), designed to permanently edit and inactivate the kallikrein B1 *(KLKB1)* gene in liver cells. It aims to prevent production of kallikrein, rebalancing the pathway that leads to bradykinin overproduction, with the goal to reduce HAE attacks. Lonvo-z is a one-time intravenous (IV) infusion administered over 2 to 4 hours in an outpatient setting.

Efficacy:
The phase 3, pivotal HAELO study, evaluating the planned commercial 50 mg lonvo-z dose is fully enrolled with results expected in April 2026.

An early phase 1/2 trial pooled the data available from 32 adults with HAE who received a 50 mg dose of lonvo-z. Prior to entering the study, on average, individuals were experiencing 3.4 attacks monthly, and 50% of those enrolled were using LTP agents. After a median follow-up of 12 months (range, 2 months to 3 years), 97% of individuals were both attack-free and were able to stop using LTP agents (LTP-free), if applicable. Seventy-five percent were both attack-free and LTP-free for a duration of at least 7 months. Details on results of the individuals receiving placebo were not available.

Safety:
Early trial data demonstrates lonvo-z was overall well-tolerated. Over half (53%) of individuals experienced infusion-related reactions that started shortly after infusion initiation and, for most, resolved in the same day. One serious adverse event occurred one-year after lonvo-z infusion in an individual with preexisting risk factors, a pulmonary embolism that resolved.

Unlike with viral vector-delivered gene therapies which elicit the development of antibodies after exposure to treatment, there may be potential for non-viral LNP delivered gene therapies like lonvo-z to be redosed in individuals who fail to respond to initial treatment. For example, in the early lonvo-z trial, 11 individuals who initially received a single dose of lonvo-z 25 mg were subsequently given a second dose of lonvo-z 50 mg, with at least two years between infusions. Of those redosed, one individual experienced a moderate elevation in a liver enzyme called aspartate aminotransferase (AST). However, overall, the manufacturer reported that the safety profile of lonvo-z 50 mg after receiving the suboptimal dose (25 mg) was consistent with the overall 50 mg population.

Product:
Lonvoguran ziclumeran (lonvo-z; also known as NTLA-2002)

Indication:
Hereditary Angioedema (HAE)

Estimated FDA approval:
2027

Therapeutic class:
Gene editing therapy

Route of administration:
Intravenous infusion

FDA designations:
Orphan; Regenerative medicine advanced therapy (RMAT)

Manufacturer:
Intellia Therapeutics

Emerging new therapies

Lonvoguran ziclumeran

Financial impact:
The price of lonvo-z is unknown. However, it could be priced similarly to other gene therapies at $1M or more per one-time treatment. If retreatment is approved, this could further increase overall financial impact.

CarelonRx view:
The availability of a new agent, administered as a one-time, outpatient infusion to prevent or reduce the frequency of HAE attacks is promising. Phase 3 results and longer-term follow-up data may help address remaining questions and clarify whether there will be clinical demand for this new treatment, given its unknown durability and the presence of well-established LTP options with proven efficacy, safety, and multiple FDA-approved therapies.

Product:
Lonvoguran ziclumeran (lonvo-z; also known as NTLA-2002)

Indication:
Hereditary Angioedema (HAE)

Estimated FDA approval:
2027

Therapeutic class:
Gene editing therapy

Route of administration:
Intravenous infusion

FDA designations:
Orphan; Regenerative medicine advanced therapy (RMAT)

Manufacturer:
Intellia Therapeutics

Emerging new therapies

Pelacarsen

Condition:

Elevated lipoprotein(a) [Lp(a)] is a genetically determined lipid disorder characterized by increased serum concentrations of Lp(a), a low-density lipoprotein (LDL) particle covalently bound to apolipoprotein(a). A large retrospective study in the United States estimated that approximately 24% of the population have Lp(a) levels exceeding 50 mg/dL. Levels in this range are associated with an increased risk of ASCVD, including coronary heart disease, stroke, and calcific aortic valve stenosis.

To date, no clinical trials have conclusively demonstrated that lowering Lp(a) levels reduces the incidence of ASCVD events. Unlike other lipid disorders, elevated Lp(a) is minimally influenced by diet, lifestyle, or conventional lipid-lowering therapies such as statins. Despite its strong association with cardiovascular (CV) morbidity and mortality, no FDA-approved therapies specifically target Lp(a) reduction.

Role in treatment:

Management of elevated Lp(a) currently focuses on comprehensive CV risk reduction, as no approved therapies directly lower Lp(a) concentrations. Standard lipid-lowering agents, including statins, ezetimibe, and fibrates, have minimal or no effect on Lp(a) concentrations, and statins may cause modest increases. Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors can reduce Lp(a) by approximately 20 to 30% although their FDA-approved indication remains limited to LDL cholesterol lowering. Niacin can lower Lp(a) by up to 30%, but its use has declined due to adverse effects and lack of demonstrated CV outcome benefit.

Lipoprotein apheresis, a blood filtration procedure similar to dialysis, can acutely lower Lp(a) by more than 60%. However, because it requires frequent, resource-intensive sessions, its use is limited to select high-risk individuals treated in specialized centers.

Pelacarsen, an antisense oligonucleotide targeting apolipoprotein(a) messenger RNA, is an investigational therapy specifically designed to reduce Lp(a) production in the liver. In clinical studies, pelacarsen has produced marked dose-dependent reductions in circulating Lp(a). It is currently being evaluated in the phase 3 Lp(a)HORIZON trial, a large global outcomes study designed to determine whether its Lp(a) lowering translates into reduced CV event rates.

Several small interfering RNA (siRNA) agents are also under investigation, including olpasiran and lepodisiran. These agents have shown similar Lp(a) reductions in early-phase studies and are administered by subcutaneous injection, differing mainly in duration of effect and dosing frequency.

Efficacy:

In a phase 2 clinical trial, pelacarsen reduced Lp(a) concentrations by up to 80% in individuals with established CV disease and high baseline levels. These reductions were consistent across doses and occurred without significant changes in other lipid parameters. The pivotal Lp(a)HORIZON outcomes trial has enrolled more than 8,000 participants and is expected to be completed in early 2026.

Product:
Pelacarsen

Indication:
Reduction of major adverse cardiovascular events in adults with established atherosclerotic cardiovascular disease (ASCVD) and elevated lipoprotein(a)

Estimated FDA approval:
Late 2026 or 2027

Therapeutic class:
Antisense oligonucleotide

Route of administration:
Subcutaneous injection

FDA designations:
Fast track

Manufacturer:
Ionis Pharmaceuticals; Novartis

Safety:

Across early clinical studies, pelacarsen has been well-tolerated. The most common side effects have been mild injection site reactions, such as redness or tenderness.

Emerging new therapies

Pelacarsen

Financial impact:
At present, no therapies are approved specifically to lower Lp(a), and the clinical and economic impact of targeting this biomarker remains uncertain. If pelacarsen demonstrates a reduction in CV events in the Lp(a)HORIZON trial, its use would likely be limited to individuals with established ASCVD and markedly elevated Lp(a), aligning with the trial population.

As an injectable antisense oligonucleotide, pelacarsen would likely be positioned within the specialty pharmacy category, with pricing and utilization management considerations similar to other RNA-based agents.

CarelonRx view:
If approved, pelacarsen would be the first therapy specifically indicated to lower Lp(a), offering a novel approach to addressing a recognized but previously difficult-to-impact CV risk factor. Early studies have shown substantial Lp(a) reductions, and if ongoing outcomes trials demonstrate meaningful reductions in events, pelacarsen could represent a marked advancement in lipid management and CV risk reduction.

Product:
Pelacarsen

Indication:
Reduction of major adverse cardiovascular events in adults with established atherosclerotic cardiovascular disease (ASCVD) and elevated lipoprotein(a)

Estimated FDA approval:
Late 2026 or 2027

Therapeutic class:
Antisense oligonucleotide

Route of administration:
Subcutaneous injection

FDA designations:
Fast track

Manufacturer:
Ionis Pharmaceuticals; Novartis

Other significant product approvals

Additional 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
Awiqli® (insulin icodec) Novo Nordisk	Type 1 diabetes mellitus; Type 2 diabetes mellitus/ subcutaneous (SC)	**Addition to class:** ultra-long-acting basal insulin analog for once-weekly dosing	March 2026	
Sasanlimab Pfizer	In combination with Bacillus Calmette-Guérin (BCG) for the treatment of BCG-naïve, high-risk non–muscle-invasive bladder cancer (NMIBC)/SC	**Addition to class:** would be first immune checkpoint inhibitor for BCG-naïve, high-risk NMIBC	Between March and May 2026	
Linerixibat GlaxoSmithKline	Cholestatic pruritus in primary biliary cholangitis (PBC)/oral	**Addition to class:** would compete with Livdelzi®	03/24/2026	
Kresladi™; RP-L201 (marnetegragene autotemcel) Rocket Pharmaceuticals	Leukocyte adhesion deficiency 1/intravenous (IV)	**First in class:** gene therapy; would be first FDA-approved treatment for this indication	03/28/2026	
Camizestrant AstraZeneca	Hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative locally advanced or metastatic breast cancer/oral	**Addition to class:** for individuals previously treated with endocrine therapy; same class as Orserdu®	Second quarter 2026	
DNL310 (tividenofusp alfa) Denali Therapeutics	Mucopolysaccharidosis II/IV	**Addition to class:** would compete with Elaprase®; ability to cross blood brain barrier	04/05/2026	

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

Table glossary:

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 December 12, 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
Vusolimogene oderparepvec Replimune	Advanced melanoma/ intratumoral	**Addition to class:** gene-based oncolytic immunotherapy; used in combination with nivolumab	04/10/2026	
BBM-H901; Dalnacogene ponparvovec Belief Biomed	Hemophilia B/IV	**Addition to class:** gene therapy would compete with Hemgenix®	04/12/2026	
MK-8591A; islatravir/ doravirine Merck	Human immunodeficiency virus 1 (HIV)-1 treatment/oral	**First in class:** two-drug regimen would compete with three-drug regimens such as Biktarvy®	04/28/2026	
CTX1301; Dexmethylphenidate Cingulate	Attention deficit hyperactivity disorder (ADHD)/oral	**Addition to class:** once-daily, rapid onset	05/31/2026	
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	
SEL-212; Rapamycin/ Pegsiticase Swedish Orphan Biovitrum AB	Gout/IV	**First in class:** every 4-week novel therapy for uncontrolled gout	06/27/2026	
Icotrokinra Johnson & Johnson	Plaque psoriasis, moderate-to-severe, ages 12 and older/oral	**First in class:** selective interleukin-23 receptor blocker	July 2026	

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* As of December 12, 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
Tavapadon Cerevel	Parkinson's disease/oral	**First in class:** submission based on data in early disease and as adjunctive to levodopa in individuals experiencing motor fluctuations	09/26/2026	
Mim8; Denecimig Novo Nordisk	Hemophilia A/SC	**Addition to class:** for individuals with or without inhibitors; potential for once-monthly dosing	09/29/2026	
Pivekimab AbbVie	Blastic plasmacytoid dendritic cell neoplasm (BPDCN)/IV	**Addition to class:** would compete with Elzonris®	09/30/2026	

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

Table glossary:

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

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* As of December 12, 2025.

The 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.

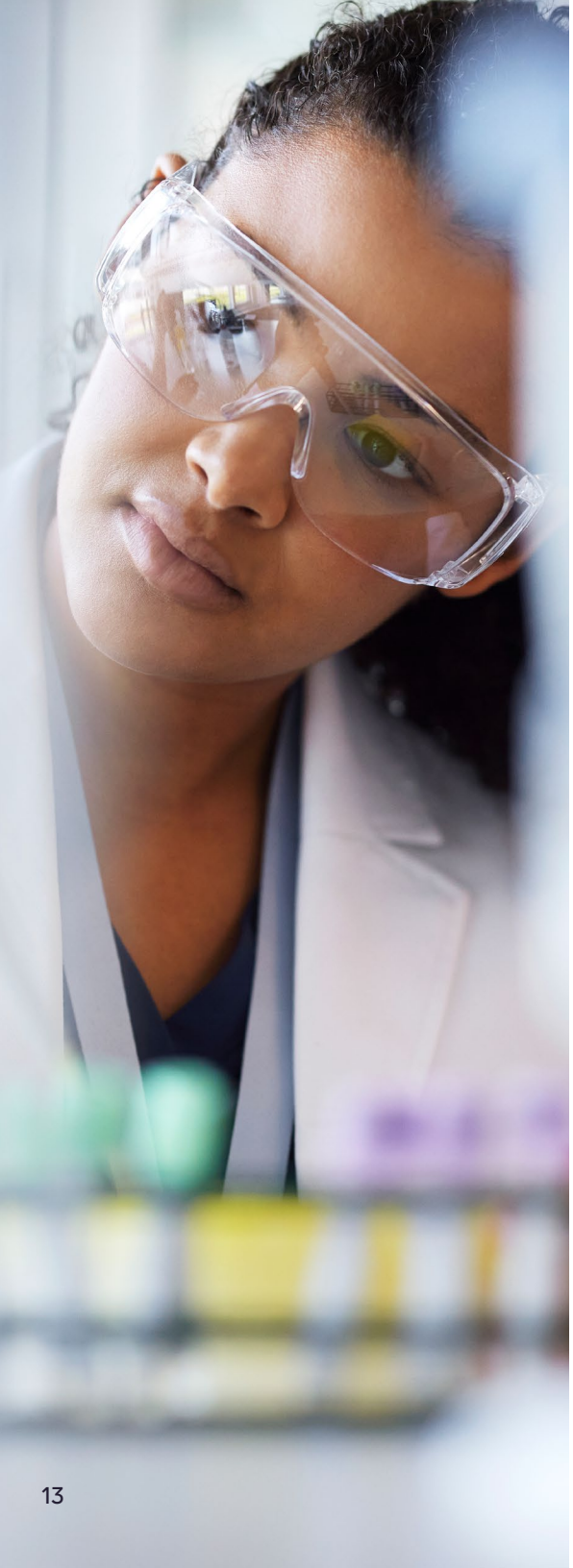
Biosimilar pipeline update

Currently, 79 biosimilar products are FDA approved in the United States which represent 20 unique reference biologic products. Recent approvals include Bosaya™, Aukelso™ (denosumab-kyqq), Enoby™, and Xtrenbo™ (denosumab-qbde) in September 2025; Eydenzelt® (aflibercept-boav), Osvyrti®, and Jubereq® (denosumab-desu) in October 2025; and Armlupeg™ (pegfilgrastim-unne) and Poherdy® (pertuzumab-dpzb) in November 2025. Sixty-two 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	01/24/2025
Avastin®	Genentech; Roche	Avzivi®	Bio-Thera Solutions; Sandoz	12/06/2023
		FKB238	Centus Biotherapeutics; AstraZeneca; Fujifilm Kyowa Kirin	Pending
Enbrel®	Amgen; Immunex	Erelzi™	Sandoz	08/30/2016
		Eticovo™	Samsung Bioepis	04/25/2019
Eylea®	Regeneron	Ahzantive®	Formycon; Santo Holding; Bioeq; Klinge Pharma	06/28/2024
		Enzeevu™	Sandoz; Hexal	08/09/2024
		Eydenzelt	Celltrion	10/02/2025
		Opuviz™	Samsung Bioepis; Biogen	05/20/2024
		Yesafili™	Momenta; Mylan; Johnson & Johnson; Biocon; Viatris	05/20/2024
		AVT06	Alvotech; Teva; Alvogen	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
Lucentis®	Roche; Genentech	LUBT010	Lupin	Pending

* As of December 12, 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*
Neulasta®	Amgen	Armlupeg	Lupin	11/28/2025
		Lapelga	Apotex; Accord; Intas	Pending
Neupogen®	Amgen	Grastofil	Apotex; Accord; Intas	Pending
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	Poherdy	Henlius; Organon	11/13/2025
Prolia®/Xgeva®	Amgen	Bosaya/Aukelso	Biocon	09/16/2025
		Enoby/Xtrenbo	Gedeon Richter/Hikma	09/26/2025
		Ospomyv™/Xbryk™	Samsung Bioepis; Samsung Biologics	02/13/2025
		Osvyrti/Jubereq	Intas; Accord	11/20/2025
		AVT03	Alvotech; Dr. Reddy's; Alvogen	Pending
		ENZ215	Enzene; Alkem Labs	Pending
		MB09	mAbxience; Insud Pharma; Fresenius Kabi; Amneal	Pending
		TVB-009P	Teva	Pending
Simponi®/Simponi Aria	Johnson & Johnson	BAT2506	Bio-Thera Solutions; Accord; Intas	Pending
Xolair®	Roche; Genentech; Novartis	Omlyclo	Celltrion	03/07/2025
		ADL-018	Kashiv Biosciences; Amneal	Pending

* As of December 12, 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 million to $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 2026*

Gene therapy/ gene-based therapy	Indication/route and frequency	Place in therapy	Estimated approval date
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 (delayed 3 months)
Marnetegragene autotemcel (marne-cel) Rocket	Leukocyte adhesion deficiency-I/ single IV infusion	First gene therapy for this indication. Uses viral vector (lentivirus).	03/28/2026 (refiled)
Vusolimogene oderparepvec Replimune	Advanced melanoma/multiple intratumoral injections	Addition to class; gene-based oncolytic immunotherapy; used in combination with nivolumab. Uses viral vector (herpes simplex virus).	04/10/2026 (refiled)
Dalnacogene ponparvovec 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 intravenous (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 December 12, 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
Bidridistrogene xeboparvovec Sarepta	Limb-girdle muscular dystrophy (LGMD) Subtype 2E/R4/single intravenous (IV)	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
DB-OTO Regeneron	Congenital hearing loss/single intracochlear infusion	First gene therapy for hearing loss due to OTOF mutations. 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
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).	2026–2027
Avalotcagene ontaparvovec Ultragenyx	Ornithine transcarbamylase (OTC) deficiency/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2026–2027

* As of December 12, 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
Casgevy™ (exagamglogene autotemcel; exa-cel) Vertex and CRISPR	Sickle cell disease/single IV infusion	Potential to expand approval to include individuals 2 to 11 years of age; will compete with HCT and chronic RBC transfusions. Uses gene editing.	2026–2027
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
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
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

* As of December 12, 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
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
Lonvoguran ziclumeran (lonvo-z) Intellia	Hereditary angioedema (HAE)/ single IV infusion	First gene therapy for this indication. Uses gene editing, delivered by lipid nanoparticles.	2027
LX2006 Lexeo	Friedreich's Ataxia Cardiomyopathy/single IV infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	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).	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
NGN-401 Neurogene	Rett Syndrome/single intracerebroventricular infusion	First gene therapy for this indication. Uses viral vector (adeno-associated virus).	2027–2028
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

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