RxOutlook®

2nd Quarter 2025

Optum Rx®

Welcome to the 2nd quarter RxOutlook Report of 2025. Optum Rx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook.

Recap of First Half 2025 - Where Are We Today?

As of May 7, the FDA's Center for Drug Evaluation and Research (CDER) has approved 10 new molecular entities in 2025. Notable drug approvals included **Journavx™ (suzetrigine)**, a first-in-class non-opioid therapy for acute pain; **Qfitlia™ (fitusiran)**, another non-factor replacement therapy for both hemophilia A and B; and **Imaavy™ (nipocalimab-aahu)**, the third neonatal Fc receptor blocker for generalized myasthenia gravis.

In addition to these drugs, the FDA's Center for Biologics Evaluation and Research (CBER) approved two gene therapies: **Zevaskyn™ (prademagene zamikeracel)**, for treatment of recessive dystrophic epidermolysis bullosa, a rare inherited skin condition, and **Encelto™ (revakinagene taroretcel-lwey)**, for macular telangiectasia type 2, a rare progressive eye disease that causes gradual loss of central vision in both eyes.

Looking Ahead to 3Q 2025

In this edition of RxOutlook, we highlight nine key products with an approval decision by the end of the 3rd quarter 2025. This includes new indications and new formulations for three existing drugs: **lenacapavir, semaglutide, and lecanemab**. Lenacapavir is currently approved under the brand name Sunlenca® for the treatment of multidrug resistant HIV-1 infection but is under FDA review for a new indication for HIV pre-exposure prophylaxis. It would be a twice-yearly dosed injection whereas current prophylaxis regimens consist of daily oral medications or an every 2-month injection. Semaglutide is currently on the market and approved across several indications (eg, type 2 diabetes and chronic weight management) and the subcutaneous formulation is under FDA review for a new indication for treatment of heart failure with preserved ejection fraction (HFpEF) in patients with obesity. Semaglutide would be the first GLP-1 receptor agonist approved for HFpEF.

In addition to these new indications, a subcutaneous formulation of lecanemab (Leqembi®) is under FDA review. Although there are no additional randomized trial data available, this would be the first self-administered beta-amyloid targeted therapy and would give an alternative to the existing intravenous products that have to be administered in a healthcare setting.

Of six novel drugs discussed in the report, two are potentially high impact drugs in areas of unmet need – **brensocatib and tolebrutinib**. Brensocatib would be the first drug approved for non-cystic fibrosis bronchiectasis, a chronic lung disease affecting about 350,000 to 500,000 people in the U.S. Tolebrutinib would be the first drug approved for slowing disability progression in individuals with non-relapsing secondary progressive multiple sclerosis (nrSPMS), a form of multiple sclerosis that is difficult to treat with existing therapies.

The other four topics in the report are orphan drugs that will be used to treat rare conditions. This includes two novel therapies for Duchenne muscular dystrophy (DMD) – **deramiocel and ataluren**. Deramiocel is a cellular therapy and would be the first treatment for cardiomyopathy-associated with DMD. Ataluren would be the first treatment specifically for patients with nonsense mutation DMD (~13% of the population). There is a high unmet need for DMD treatments, with the current landscape limited by agents mostly approved under accelerated approval (eg, exon skipping therapies) and with very limited efficacy data. These treatments are also mutation-specific and only cover approximately 30% of the DMD population.

Finally, **donidalorsen** and **paltusotine** would be novel therapies entering competitive treatment landscapes for hereditary angioedema and acromegaly, respectively. Donidalorsen will be competing with other branded HAE prophylaxis agents, including injectable Haegarda® (C1 esterase inhibitor), Cinryze® (C1 esterase inhibitor), and Takhzyro® (lanadelumab-flyo), as well as orally administered Orladeyo® (berotralstat). Paltusotine will primarily be competing with injectable drugs such as octreotide and lanreotide, which have formulations available generically.

Key FDA approval decisions expected by the end of the 3rd quarter 2025

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Lenacapavir	Gilead	HIV pre-exposure prophylaxis	6/19/2025
Wegovy (semaglutide)	Novo Nordisk	Heart failure and obesity	3Q 2025
Brensocatib	Insmed	Bronchiectasis	8/12/2025
Donidalorsen	Ionis	Hereditary angioedema*	8/21/2025
Leqembi SC (lecanemab)	Eisai/Biogen	Alzheimer's disease	8/31/2025
Deramiocel	Capricor	Duchenne muscular dystrophy*	8/31/2025
Translarna (ataluren)	PTC Therapeutics	Duchenne muscular dystrophy*	3Q 2025
Paltusotine	Crinetics	Acromegaly*	9/25/2025
Tolebrutinib	Sanofi	Multiple sclerosis	9/28/2025

* Orphan Drug Designation

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 3rd quarter 2025.

Read more

Extended Brand Pipeline Forecast

This supplemental table provides a summary of developmental drugs, including both traditional and specialty medications that may be approved in the upcoming two years.

Read more

Key Pending Indication Forecast

This supplemental table provides a summary of key new indications that are currently under review by the FDA and may be approved in the upcoming 12 months.

Read more

Extended Generic Pipeline Forecast

This section provides a summary of upcoming first-time generic drugs and biosimilars that may be approved in the upcoming two years.

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 3rd quarter 2025 may appear in future reports; however, for those who need an initial look at the larger pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Read more

Getting acquainted with pipeline forecast terms

Clinical trial phases

Phase I trials	Researchers test an experimental drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.
Phase II trials	The experimental study drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.
Phase III trials	The experimental study drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
Phase IV trials	Post marketing studies delineate additional information including the drug's risks, benefits, and optimal use.

Pipeline acronyms

ANDA	Abbreviated New Drug Application
BLA	Biologic License Application
CRL	Complete Response Letter
FDA	Food and Drug Administration
MOA	Mechanism of Action
NME	New Molecular Entity
NDA	New Drug Application
sBLA	Supplemental Biologic License Application
sNDA	Supplemental New Drug Application
OTC Drugs	Over-the-Counter Drugs
PDUFA	Prescription Drug User Fee Act
REMS	Risk Evaluation and Mitigation Strategy

Detailed Drug Insights

Lenacapavir (Brand Name: TBD)

Manufacturer: Gilead
Regulatory Designations: Breakthrough Therapy
Expected FDA decision: June 19, 2025

Therapeutic use

Lenacapavir is under review for pre-exposure prophylaxis (PrEP) to reduce the risk of sexually acquired HIV-1 infection in people who are at risk for HIV-1 acquisition.

Lenacapavir is currently approved under the brand name Sunlenca® for the treatment of multidrug resistant HIV-1 infection in treatment-experienced adults failing their current antiretroviral therapy.

When taken as prescribed, PrEP is highly effective in preventing infection in HIV-negative, at-risk individuals. In the U.S., HIV affects approximately 1.2 million people. The incidence of HIV infections has decreased by 12% from 2018 to 2022. This downward trend is partially attributed to the increase in PrEP therapy.

Clinical profile

Lenacapavir is a multistage HIV-1 capsid inhibitor that inhibits HIV replication by binding between p24 capsid subunits at various essential steps of the viral life cycle.

Pivotal trial data:
The efficacy of lenacapavir was evaluated in PURPOSE 1 and PURPOSE 2, two Phase 3, randomized, double-blind, active-controlled studies in HIV-negative patients with high risk of acquiring HIV. Patients were randomized to receive lenacapavir or emtricitabine-tenofovir disoproxil fumarate (F/TDF), along with matching placebos. The primary endpoint in both studies, assessed when 50% of patients had received treatment for one year, was HIV incidence with lenacapavir treatment compared to the incidence at background. The secondary endpoint compared HIV incidence between lenacapavir and F/TDF.

PURPOSE 1 was conducted in 5,338 cisgender female patients between 16 to 25 years of age from areas of significant HIV incidence. At the time of analysis, HIV incidence with lenacapavir (0 cases per 100 person-years, or 0%) was significantly lower compared to the 2.41% background incidence (incidence rate ratio of 0.00, 95% CI: 0.00, 0.04; $p < 0.001$) and the 1.69% incidence with F/TDF (incidence rate ratio of 0.00, 95% CI: 0.00, 0.10; $p < 0.001$).

PURPOSE 2 was conducted in 3,265 patients 16 years or older from areas of significant HIV transmission who identified as cisgender gay, bisexual, or other men; transgender; or nonbinary. At the time of analysis, HIV incidence with lenacapavir was 0.1%, which was significantly lower than both the 2.37% background incidence (incidence rate ratio of 0.04, 95% CI: 0.01, 0.18; $p < 0.001$) and the 0.93% incidence with F/TDF (incidence rate ratio of 0.11, 95% CI: 0.02, 0.51; $p = 0.002$).

What you need to know:

Proposed Indication: PrEP to reduce the risk of sexually acquired HIV-1 infection in people who are at risk for HIV-1 acquisition

Mechanism: HIV capsid inhibitor

Efficacy: Annual HIV incidence:

- PURPOSE 1: 0% with lenacapavir vs. 1.69% with F/TDF
- PURPOSE 2: 0.1% with lenacapavir vs. 0.93% with F/TDF

Common AEs: Injection site reactions

Dosing: SC every 6 months; oral lead-in with initial injection

Why it Matters: First twice-yearly HIV PrEP treatment, demonstrated highest reductions in HIV incidence vs. alternatives, potential once-yearly administration

Important to Note: Low-cost generic alternative (Truvada), requires healthcare provider administration

Estimated Cost: ~$19,500 per dose (based on current pricing of Sunlenca)

Lenacapavir (continued...)

Safety:
The most common adverse events with lenacapavir use were injection site reactions.

Dosing:
In the pivotal trials, lenacapavir was administered as two subcutaneous (SC) injections every 6 months. An initial loading dose of oral lenacapavir over 2 days was administered with the first injection.

Competitive environment

If approved for HIV PrEP, lenacapavir will join Gilead's once daily oral agents, Truvada® (emtricitabine/tenofovir disoproxil fumarate) and Descovy® (emtricitabine/tenofovir alafenamide fumarate), and ViiV's bimonthly intramuscular agent, Apretude (cabotegravir), as options for HIV prevention. Of the available treatment options, only Truvada is currently available generically.

Lenacapavir will be the first twice-yearly HIV PrEP treatment. Lenacapavir's extended dosing interval offers the potential to improve medication adherence, which is crucial for effective HIV prevention and remains a challenge with alternative PrEP agents. The data for lenacapavir as PrEP are promising, demonstrating the highest reductions in HIV incidence compared indirectly to the current treatment options. In the second half of 2025, Gilead will be pursuing a Phase 3 study investigating the efficacy of a once-yearly intramuscular lenacapavir formulation for HIV PrEP.

Lenacapavir requires administration by a healthcare provider, which will result in a shift in PrEP utilization from the Pharmacy Benefit to the Medical Benefit.

The Wholesale Acquisition Cost (WAC) for Sunlenca is approximately $19,500 per dose.

Semaglutide (Brand Name: Wegovy®)

Manufacturer: Novo Nordisk
Expected FDA decision: 3Q 2025

Therapeutic use

Wegovy is under review for the treatment of adults with heart failure with preserved ejection fraction (HFpEF) and obesity.

Wegovy is currently approved to reduce the risk of major adverse cardiovascular events (cardiovascular death, non-fatal myocardial infarction, or non-fatal stroke) in adults with established cardiovascular disease and either obesity or overweight; and to reduce excess body weight and maintain weight reduction long term in adults and pediatric patients aged 12 years and older with obesity and adults with overweight in the presence of at least one weight-related comorbid condition.

Semaglutide is also approved under the brand name Ozempic® to improve glycemic control in adults with type 2 diabetes mellitus; to reduce the risk of major adverse cardiovascular events (cardiovascular death, non-fatal myocardial infarction or non-fatal stroke) in adults with type 2 diabetes mellitus and established cardiovascular disease; and to reduce the risk of sustained estimated glomerular filtration rate (eGFR) decline, end-stage kidney disease, and cardiovascular death in adults with type 2 diabetes mellitus and chronic kidney disease.

Clinical profile

Wegovy is a glucagon-like peptide 1 (GLP-1) receptor agonist.

What you need to know:

Proposed Indication: Treatment of adults with HFpEF and obesity

Mechanism: GLP-1 receptor agonist

Efficacy:

- Change from baseline in KCCQ-CSS: 13.7 to 16.6 with Wegovy vs. 6.4 to 8.7 with placebo
- Composite of cardiovascular death or heart failure events (post-hoc pooled analysis): 5.4% with Wegovy vs. 7.5% with placebo

Common AEs: Gastrointestinal symptoms

Dosing: SC once every week

Why it Matters: First GLP-1 receptor agonist for heart failure, potential as an alternative or add-on to SGLT2 inhibitors for HFpEF

Important to Note: Data is primarily limited to patients with obesity (BMI ≥ 30 kg/m²), narrower indication compared to SGLT2 inhibitors

WAC: ~$16,200 per year (based on current pricing of Wegovy)

Pivotal trial data:
The efficacy of Wegovy was evaluated in STEP HFpEF and STEP HFpEF-DM, two randomized, double-blind, placebo-controlled studies in patients with HFpEF and obesity. STEP HFpEF was conducted in 529 patients without type 2 diabetes and STEP HFpEF-DM was conducted in 616 patients with type 2 diabetes. The dual primary endpoints were the change from baseline in the Kansas City Cardiomyopathy Questionnaire clinical summary score (KCCQ-CSS; scores range from 0 to 100, with higher scores indicating fewer symptoms and physical limitations) and the change in body weight.

In STEP HFpEF, the mean change in the KCCQ-CSS was 16.6 points with Wegovy vs. 8.7 points with placebo (estimated difference 7.8 points, 95% CI: 4.8, 10.9; $p < 0.001$), and the mean percentage change in body weight was −13.3% with Wegovy vs. −2.6% with placebo (estimated difference −10.7, 95% CI: −11.9, −9.4; $p < 0.001$).

In STEP HFpEF-DM, the mean change in the KCCQ-CSS was 13.7 points with Wegovy vs. 6.4 points with placebo (estimated difference 7.3 points; 95% CI: 4.1, 10.4; $p < 0.001$), and the mean percentage change in body weight was −9.8% with Wegovy vs. −3.4% with placebo (estimated difference −6.4; 95% CI: −7.6, −5.2; $p < 0.001$).

Semaglutide (continued...)

In addition, a post-hoc, pooled participant-level analysis was conducted of four randomized, placebo-controlled trials – SELECT, FLOW, STEP-HFpEF, and STEP-HFpEF DM, to examine the effects of semaglutide on heart failure events. The SELECT trial enrolled participants with atherosclerotic cardiovascular disease and overweight or obesity, and the FLOW trial enrolled participants with type 2 diabetes and chronic kidney disease. This analysis included all patients from the STEP-HFpEF trials and those with an investigator-reported history of HFpEF from SELECT and FLOW. Across the four trials, 3,743 patients had a history of HFpEF. The main outcomes for this pooled analysis were the composite endpoint of time to cardiovascular death or first worsening heart failure event (defined as hospitalization or urgent visit due to heart failure), time to first worsening heart failure event, and time to cardiovascular death.

Semaglutide reduced the risk by 31% for the combined endpoint of cardiovascular death or heart failure events (5.4% in the semaglutide group had events vs. 7.5% in the placebo group; hazard ratio [HR] 0.69, 95% CI: 0.53, 0.89; p = 0.0045). Semaglutide also reduced the risk of worsening heart failure events (2.8% vs. 4.7%; HR 0.59, 95% CI: 0.41, 0.82; p = 0.0019). There was no significant difference in the risk of cardiovascular death alone.

Safety:
The most common adverse event with Wegovy use was gastrointestinal symptoms.

Dosing:
In the pivotal trials, Wegovy was administered SC once every week.

Competitive environment

The current standard of care for treatment of HFpEF are diuretics and sodium-glucose co-transporter 2 (SGLT2) inhibitors (ie, Farxiga® [dapagliflozin] and Jardiance® [empagliflozin]). Other treatment options, such as mineralocorticoid receptor antagonists, Entresto® (sacubitril-valsartan), and other renin-angiotensin-system inhibitors can be used, particularly in patients with comorbid conditions. Despite these options, there is still an unmet need for additional therapies for this population.

If approved, Wegovy would be the first GLP-1 receptor agonist approved for HFpEF. In two Phase 3 trials in patients with HFpEF, Wegovy demonstrated improvements in heart failure symptoms and physical limitations vs. placebo. A post-hoc pooled analysis of four trials with semaglutide also demonstrated an improvement in reducing the risk of heart failure events.

A key limitation for Wegovy is that it was primarily evaluated in HFpEF patients with obesity (BMI > 30 kg/m²). This will reduce its use in HFpEF compared to SGLT2 inhibitors, which can be used in all patients with the disease, regardless of obesity status. SGLT2 inhibitors are also approved in patients with heart failure with reduced ejection fraction.

Wegovy could potentially be used as an add-on therapy to SGLT2 inhibitors, particularly in patients with type 2 diabetes (33% of patients were on concomitant SGLT2 inhibitors in the STEP HFpEF-DM trial).

For reference, the WAC for Wegovy is approximately $16,200 per year.

Brensocatib (Brand Name: To be determined)

Manufacturer: Insmed
Regulatory designation: Breakthrough Therapy
Expected FDA decision: August 12, 2025

Therapeutic use

Brensocatib is under review for the treatment of patients with non-cystic fibrosis bronchiectasis.

Bronchiectasis is a chronic lung disease where the bronchi (airway walls) become irreversibly thickened and damaged. As the bronchi become increasingly damaged, mucus builds up in the lungs, which can lead to a cycle of inflammation and infection. The most common symptoms associated with the disease are chronic cough, excessive sputum production, shortness of breath, and recurrent respiratory infections.

The cause of bronchiectasis is often unknown, but many conditions have been linked or can trigger the disease, including autoimmune diseases and chronic obstructive pulmonary disease (COPD). Bronchiectasis affects 350,000 to 500,000 adults in the U.S., and the risk of developing bronchiectasis increases with age.

What you need to know:

Proposed Indication: Treatment of patients with non-cystic fibrosis bronchiectasis

Mechanism: DPP1 inhibitor

Efficacy: Annualized rate of pulmonary exacerbations: 1.015 to 1.036 with brensocatib vs. 1.286 with placebo

Common AEs: Nasopharyngitis, cough, headache

Dosing: Oral once daily

Why it Matters: Potentially the first therapy for non-cystic fibrosis bronchiectasis, well tolerated, unmet need

Important to Note: Lack of robust data for prevention of severe pulmonary exacerbations (eg, requiring hospitalization), affects an older population (average age in the Phase 3 study was 60 years)

Estimated Cost: $40,000 to $96,000 per year

Clinical profile

Brensocatib is a reversible inhibitor of dipeptidyl peptidase 1 (DPP1). DPP1 is an enzyme responsible for activating neutrophil serine proteases (NSPs). In chronic inflammatory lung diseases, neutrophils accumulate in the airways and result in excessive active NSPs that cause lung destruction and inflammation. Brensocatib may decrease the damaging effects of inflammatory diseases such as bronchiectasis by inhibiting DPP1 and its activation of NSPs.

Pivotal trial data:
The efficacy of brensocatib was evaluated in ASPEN, a randomized, double-blind, placebo-controlled study in 1,680 adult and 41 adolescent patients with bronchiectasis. Patients were randomized to brensocatib 10 mg, brensocatib 25 mg, or placebo for 52 weeks. The primary endpoint was the rate of adjudicated pulmonary exacerbations over 52 weeks. Pulmonary exacerbations were defined as the presence of ≥ 3 symptoms for at least 48 hours, resulting in a physician's decision to prescribe systemic antibiotics.

The annualized rate of pulmonary exacerbations was 1.015, 1.036, and 1.286 with brensocatib 10 mg, brensocatib 25 mg, and placebo, respectively. This represented a 21.1% ($p = 0.0019$) and 19.4% ($p = 0.0046$) risk reduction between brensocatib 10 mg and 25 mg vs. placebo. Both doses also demonstrated numerical reductions in the rate of severe pulmonary exacerbations (defined as those requiring intravenous antibiotics and/or hospitalization), but this was not statistically significant.

Brensocatib (continued...)

Safety:
The most common adverse events with brensocatib use were nasopharyngitis, cough, and headache.

Dosing:
In the pivotal trial, brensocatib was administered orally once daily.

Competitive environment

If approved, brensocatib would be the first targeted therapy for non-cystic fibrosis bronchiectasis, a condition with a significant unmet need. The current standard of care is managing the underlying conditions, mucus thinning medications and nebulizer treatments, and antibiotics for infections.

The trial results for brensocatib were promising, with an improvement in the overall rate of pulmonary exacerbations compared to placebo. It was also well tolerated, with rates of adverse events comparable to placebo.

The difference in severe pulmonary exacerbations, a secondary endpoint in the Phase 3 trial, did favor brensocatib numerically when compared to placebo, but the difference was not statistically significant. The study was not powered for this endpoint, but more robust data for reductions in hospitalization would have added to the value proposition for brensocatib.

Finally, while bronchiectasis can develop at any age, the risk increases significantly in older adults. In the ASPEN trial, the average age was 60 years.

The estimated WAC for brensocatib is $40,000 to $96,000 per year.

Donidalorsen (Brand Name: To be determined)

Manufacturer: Ionis Pharmaceuticals
Regulatory designations: Orphan Drug
Expected FDA decision: August 21, 2025

Therapeutic use

Donidalorsen is under review for prophylactic treatment to prevent hereditary angioedema (HAE) attacks in adult and pediatric patients 12 years and older.

HAE is a rare genetic condition characterized by recurrent spontaneous episodes, or attacks, of severe fluid accumulation and swelling in the extremities, face, stomach, or airways. The potentially life-threatening attacks are managed with medications used as treatment (for acute episodes) or prophylaxis (for prevention of episodes).

It is estimated that approximately 7,000 people in the U.S. have HAE.

Clinical profile

Donidalorsen is a prekallikrein (PKK) inhibitor that selectively binds to PKK mRNA to reduce the production of plasma PKK, a precursor to kallikrein. Plasma kallikrein promotes the release of bradykinin, which is responsible for the swelling in HAE when overactive.

What you need to know:

Proposed Indication: Prophylactic treatment to prevent HAE attacks in adult and pediatric patients 12 years and older

Mechanism: PKK mRNA inhibitor

Efficacy: HAE attacks per month at Week 25: 0.44 with monthly donidalorsen vs. 2.26 with placebo (81% reduction); 1.02 with bimonthly donidalorsen (55% reduction)

Common AEs: Injection site reactions

Dosing: SC monthly or bimonthly

Why it Matters: First-in-class RNA-targeted HAE therapy, reduced dosing frequency vs. alternatives

Important to Note: Lack of direct head-to-head data vs. competitors, Cinryze, Haegarda, and Takhzyro indicated for broader age group, oral alternative available (Orladeyo), potential competitor to enter market in June 2025 (garadacimab)

Estimated Cost: ~$500,000 per year (based on average pricing of current therapies)

Pivotal trial data:
The efficacy of donidalorsen was evaluated in OASIS-HAE, a Phase 3, randomized, double-blind study in 90 patients 12 years or older with HAE. Patients were randomized to receive donidalorsen or placebo. The primary endpoint was the monthly rate of HAE attacks from Weeks 1 to 25.

At Week 25, the rate of HAE attacks was significantly lower in patients treated with monthly and bimonthly donidalorsen (0.44 and 1.02, respectively) compared to placebo (2.26), with relative risk reduction rates of 81% (95% CI: 65%, 89%; $p < 0.001$) with monthly dosing and 55% (95% CI: 22%, 74%; $p = 0.004$) with bimonthly dosing.

Safety:
In the pivotal trial, the most common adverse event with donidalorsen use was injection site reactions.

Dosing:
In the pivotal trial, donidalorsen was self-administered via SC injection every 4 or 8 weeks.

Donidalorsen (continued...)

Competitive environment

If approved, donidalorsen will join a competitive market as a first-in-class RNA-targeted HAE therapy. Current marketed HAE prophylaxis agents include CSL's twice weekly SC Haegarda® (C1 esterase inhibitor); Takeda's twice weekly IV Cinryze® (C1 esterase inhibitor) and biweekly SC Takhzyro® (lanadelumab-flyo); and BioCryst's once daily oral Orladeyo® (berotralstat). A key differentiator for donidalorsen compared to its competitors is the reduced frequency of administration.

In an open-label extension trial (OASISplus), data suggested that patients who switched from existing therapies to donidalorsen experienced reductions in HAE attacks. However, direct head-to-head trials are needed to establish comparative efficacy. Donidalorsen's target population is limited compared to the other SC agents, which are indicated for a broader age group (Cinryze and Haegarda for 6 years and up, Takhzyro for 2 years and up). Additionally, Orladeyo may be preferable for patients with needle hesitancies or phobias.

Prior to the FDA's decision for donidalorsen in the third quarter, the market may become even more competitive. CSL's novel factor XIIa-inhibitory monoclonal antibody garadacimab, which offers similar dosing convenience with once monthly SC administration, is expected to receive a decision from the FDA in June 2025.

For reference, the average WAC of HAE prophylaxis therapies is approximately $500,000 per year.

Lecanemab (Brand Name: Leqembi SC)

Manufacturer: Eisai/Biogen
Expected FDA decision: August 31, 2025

Therapeutic use

Leqembi SC is under review for the maintenance treatment of Alzheimer's disease in patients with mild cognitive impairment (MCI) or mild dementia.

Leqembi is currently available as an IV formulation.

Alzheimer's disease is an irreversible, progressive brain disorder that slowly destroys memory and cognition. MCI is usually the first sign of Alzheimer's disease which then progresses to dementia related to Alzheimer's disease (further classified as mild, moderate, or severe dementia). The disease is characterized by changes in the brain, including the abnormal accumulation of toxic amyloid beta plaque.

Alzheimer's disease is the most common form of dementia. It affects about 6 million people in the U.S., and it is the 5th leading cause of death among adults aged 65 years or older.

Clinical profile

Lecanemab is a monoclonal antibody that binds to soluble beta amyloid aggregates (oligomers and protofibrils) with high selectivity.

What you need to know:

Proposed Indication: Maintenance treatment of Alzheimer's disease in patients with MCI or mild dementia

Mechanism: Amyloid beta-directed antibody

Safety: Consistent with safety of IV Leqembi

Dosing: SC once every week

Why it Matters: First self-administered product in the class, eliminates the need for IV infusions for long-term maintenance therapy

Important to Note: Modest efficacy (based on results for Leqembi IV), SC formulation does not eliminate the risk of ARIA adverse events, requires IV therapy for initiation and then weekly SC maintenance therapy

Estimated Cost: ~$26,500 per year (based on pricing for IV Leqembi)

Pivotal trial data:
The FDA submission for the SC formulation of Leqembi is supported by an open-label extension trial comparing the pharmacokinetic and pharmacodynamic profile to the IV formulation of Leqembi. SC Leqembi demonstrated bioequivalence to the IV formulation with a similar pharmacokinetic profile. The pharmacodynamic profiles were also similar, with SC Leqembi providing comparable amyloid removal to the IV formulation at 6 months of treatment.

Safety:
The most common adverse events with Leqembi use were infusion-related reactions, amyloid related imaging abnormality (ARIA)-microhemorrhages, ARIA-edema/effusion, and headache.

Dosing:
The SC formulation of Leqembi is administered once every week.

Lecanemab (continued...)

Competitive environment

If approved, Leqembi SC would be the first self-administered beta-amyloid targeted therapy. The primary advantage to an available self-administered product would be improved patient access and eliminating the need for IV infusions long-term for maintenance therapy. This would shift utilization for these drugs from the Medical to the Pharmacy benefit and shift drug spend from Medicare Part B to Medicare Part D.

Leqembi SC has the same limitations as are known with the overall beta-amyloid class and the IV formulation of Leqembi. Eisai did not conduct additional randomized Phase 3 trials for the SC formulation and like the IV formulation, the clinical benefit is expected to be modest. The SC formulation also does not eliminate the risk of ARIA adverse events which have been a limiting factor for initiating patients on therapy with this class of drugs. ARIA adverse events can result in serious brain swelling or hemorrhaging, which drives the need for routine MRI monitoring with these drugs.

The SC formulation would be used after patients are treated with an initial period of IV administered Leqembi; however, Eisai is expected to file for FDA approval for initiating therapy with SC Leqembi in the first half of 2026.

For reference, the WAC for Leqembi is approximately $26,500 per year.

Deramiocel (Brand Name: To be determined)

Manufacturer: Capricor Therapeutics
Regulatory Designations: Orphan Drug
Expected FDA decision: August 31, 2025

Therapeutic use

Deramiocel is under review for the treatment of Duchenne muscular dystrophy (DMD) cardiomyopathy.

DMD is a rare genetic disorder that affects mainly males. Patients with DMD experience progressive muscle atrophy from inadequate production of the structural protein dystrophin. The lack of functional dystrophin causes cardiac, skeletal, and respiratory muscle cell death. Patients usually experience symptoms by age 3, become non-ambulatory by their early teens, lose upper limb function in their late teens, and progress to pulmonary or cardiac failure by their 20s. The leading cause of DMD mortality is heart failure from cardiomyopathy, with a median mortality age of 30 years.

Approximately 15,000 to 20,000 patients in the U.S. are affected by DMD.

Clinical profile

Deramiocel is a cardiac stem cell-based therapy that regulates the pro-inflammatory state of DMD through immunomodulatory, antifibrotic, and regenerative activity.

What you need to know:

Proposed Indication: Treatment of DMD-associated cardiomyopathy

Mechanism: Cardiac stem cell therapy

Efficacy:

- Change in mid-level PUL score (primary endpoint): -0.8 with deramiocel vs. -3.4 with placebo
- Change in LVEF (secondary endpoint): 0.1% with deramiocel vs. -3.9% with placebo

Common AEs: Hypersensitivity

Dosing: IV once every 3 months

Why it Matters: First-in-class treatment for DMD-associated cardiomyopathy, first therapy to demonstrate benefit in skeletal and cardiac muscle disease progression, addresses broad DMD population, high unmet need

Important to Note: Small study population, potential new drug spend as add-on therapy, requires health care provider administration

Estimated Cost: ≥ $750,000 per year (based on average pricing of exon-skipping therapies)

Pivotal trial data:
The efficacy of deramiocel was evaluated in HOPE-2 Phase 2 and OLE studies in male patients with late stage DMD.

In the Phase 2, randomized, double-blind study, 20 patients 10 years and older were randomized to receive deramiocel or placebo, in addition to standard therapy, for 12 months. The primary endpoint was the change from baseline in mid-level elbow Performance of Upper Limb (PUL) version 1.2, an instrument that scores arm motility from no function (lowest score) to full function (highest score). Cardiac function was assessed as a secondary endpoint. At Month 12, treatment with deramiocel demonstrated favorable upper limb function compared to placebo, with a mean PUL score change of -0.8 and -3.4 (treatment difference: 2.6; $p = 0.014$), respectively. Deramiocel treatment also significantly reduced ventricular ejection fraction (LVEF) decline at Month 12, with mean LVEF change from baseline of 0.1% with deramiocel and -3.9% with placebo (treatment difference: 4%; $p = 0.0022$).

The ongoing OLE study includes 13 non-ambulatory male patients taking deramiocel. The primary endpoint was the change from baseline at Month 12 in PUL version 2.0. At Month 12, the mean PUL score change compared to the original 1-year decline rate of the Phase 2 study population was -1.8 (SD: 3.1; $p = 0.002$). At Month 36, PUL score changes were smaller with deramiocel (-4.1) than with an external comparator (-7.8; treatment difference: 3.7; $p < 0.001$). At Month 36, the mean LVEF increased by 1.2%.

Deramiocel (continued...)

Safety:
In the Phase 2 study, three patients experienced hypersensitivity reactions, one of which was severe, requiring epinephrine and resulting in the implementation of a pretreatment regimen. At Month 12 of the OLE, 2 patients experienced severe AEs requiring medical intervention, but there were no life-threatening AEs or death.

Dosing:
In the pivotal trials, deramiocel was administered once every 3 months as an intravenous (IV) infusion.

Competitive environment

There is a high unmet need for DMD treatments, with the current landscape limited by agents approved under accelerated approval (ie, continuous approval dependent on further data) and with limited efficacy data. The exon skipping therapies (manufacturing partner NS Pharma's Viltepso® [viltolarsen] and Sarepta's Exondys 51® [eteplirsen], Amondys 45® [casimersen], and Vyondys 53® [golodirsen]) are mutation-specific and cover approximately 30% of the DMD population. Sarepta's one-time gene therapy, Elevidys® (delandistrogene moxeparvovec-rokl), is indicated for patients 4 years and older. If approved, deramiocel will be a first-in-class treatment for DMD-associated cardiomyopathy, as well as the first DMD therapy to address skeletal myopathy and cardiomyopathy, without mutation-specific limitations. As cardiomyopathy is the main driver of mortality, deramiocel will address a high unmet need if approved.

The population size of HOPE-2 was small. Results from the ongoing Phase 3 study of 102 patients (set to complete at the end of 2026), will provide additional data for deramiocel, and if positive, solidify its market potential. Additionally, because of its unique mechanism of action, deramiocel has the potential to be an add-on therapy to existing treatments.

Capricor is expected to list deramiocel at a price similar to or higher than exon skipping therapies. The average WAC of exon skipping therapies is approximately $750,000 per year.

Ataluren (Brand Name: Translarna™)

Manufacturer: PTC Therapeutics
Regulatory Designations: Orphan Drug
Expected FDA decision: 3Q 2025

Therapeutic use

Translarna is under review for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD).

Nonsense mutations result in the production of non-functional, incomplete dystrophin proteins. Approximately 13% of the 15,000 to 20,000 DMD cases in the U.S. are nmDMD.

Clinical profile

Translarna is a protein restoration therapy that allows readthrough of the nonsense mutation during RNA translation to produce full-length, functional dystrophin.

Pivotal trial data:
The efficacy of Translarna was evaluated in Study 041 and the STRIDE registry in male patients with nmDMD.

Study 041, a Phase 3, randomized, double-blind study, included 359 patients 5 years and older who were randomized to receive Translarna or placebo, along with standard therapy, for 72 weeks. The primary endpoint was the change from baseline in 6-minute walk test (6MWT) in a primary analysis cohort of 185 patients who were 7 to 16 years old with pronounced functional impairment. At Week 72, the 8.3-meter difference in 6MWT distance and 11% 6MWT change rate between Translarna (-81.8 meters and -1.14 meters/week) and placebo (-90.1 meters and -1.25 meters/week) were not statistically significant (p = 0.3626). In a secondary analysis of the intent-to-treat (ITT) population (ie, all patients randomized and received at least 1 dose), Translarna slowed 6MWT decline rate by 20% (rate of change: -0.74 meters/week with Translarna compared to -0.94 meters/week with placebo; p = 0.0248). Patients on Translarna demonstrated less decline compared to placebo, with 6MWT distance reductions of 53.0 and 67.4 meters (treatment difference: 14.4 meters; p = 0.0248), respectively.

The STRIDE patient registry is an ongoing observational study collecting real-world data of patients with nmDMD taking Translarna. An interim analysis at the 7-year mark evaluated 261 patients from STRIDE and 261 matched patients (from the Cooperative International Neuromuscular Research Group Duchenne Natural History Study) taking standard of care. Treatment with Translarna demonstrated a 4-year delay in loss of ambulation (median age at loss of ambulation: 17.0 years with Translarna, 13.0 years with standard of care; p < 0.0001). Patients taking Translarna had a later age to pulmonary decline, as represented by forced vital capacity < 60%, of 17.7 years compared to standard of care of 15.9 years (treatment difference: 1.8 years; p = 0.0021).

What you need to know:

Proposed Indication: Treatment of nmDMD

Mechanism: Protein restoration therapy

Efficacy: 6MWT:

- Primary analysis population (pronounced impairment): Distance change: -81.8 meters with Translarna vs. -90.1 meters with placebo (not significant); rate of change: -1.14 meters/week vs. -1.25 meters/week with placebo (not significant)
- Secondary analysis population (ITT): Distance change: -53.0 meters with Translarna vs. -67.4 meters with placebo; rate of change: -0.74 meters/week with Translarna vs. -0.94 meters/week with placebo

Common AEs: Abdominal pain, constipation, diarrhea, increased lipids, increased triglycerides

Dosing: Granules for oral suspension taken three times daily

Why it Matters: First-in-class therapy for nmDMD, high unmet need

Important to Note: Narrow indication, mixed study results, failed to meet primary endpoint in the Phase 3 trial

Ataluren (continued...)

Safety:
The frequency of adverse events was similar between Translarna and placebo in Study 041, with no serious adverse events related to Translarna. In the STRIDE registry, the most common treatment-related AEs were gastrointestinal (ie, abdominal pain, constipation, and diarrhea) or metabolic in nature (increased triglycerides and lipids).

Dosing:
In the pivotal trials, Translarna was dosed by weight and administered as an oral suspension three times a day.

Competitive environment

Translarna would be a first-in-class therapy for nmDMD. This is a subpopulation of DMD with no FDA approved targeted therapy and represents an area of high unmet need.

This is PTC's third FDA submission for Translarna, with the FDA not approving previous applications because of mixed efficacy results. Previous studies yielded inconsistent results, failing primary endpoints but achieving certain secondary endpoints, which was also observed with the more recent Study 041 trial.

The target population for Translarna is expected to be small given the rarity of DMD and because Translarna would be limited to patients with the nonsense mutations (only 13% of patients).

Paltusotine (Brand Name: To be determined)

Manufacturer: Crinetics Pharmaceuticals
Regulatory designation: Orphan Drug
Expected FDA decision: September 25, 2025

Therapeutic use

Paltusotine is under review for the treatment and long-term maintenance therapy of acromegaly in adults.

Acromegaly is a rare debilitating disorder of slow but progressive bone and soft tissue enlargement. This abnormal growth is caused by an excess of growth hormones, which stimulate the release of insulin-like growth factor-1 (IGF-1), a hallmark of acromegaly correlated with disease activity. Symptoms increase in prominence and severity with age, altering physical appearances and predisposing affected individuals to bone, joint, cardiovascular, neurological, or respiratory disease. If left untreated, acromegaly can lead to premature mortality.

Acromegaly is often unrecognized and underdiagnosed, but approximately 27,000 people in the U.S. have diagnosed acromegaly.

Clinical profile

Paltusotine is a selective somatostatin receptor type 2 (SST2) nonpeptide agonist that acts as a somatostatin receptor ligand (SRL) to inhibit pituitary growth hormone release.

What you need to know:

Proposed Indication: Treatment and long-term maintenance of acromegaly in adults

Mechanism: SST2 nonpeptide agonist

Efficacy: Normalized IGF-1:

- PATHFNDR-1: 83% with paltusotine vs. 4% with placebo (difference: 79%)
- PATHFNDR-2: 56% with paltusotine vs. 5% with placebo (difference: 51%)

Common AEs: Diarrhea, abdominal pain, and nausea

Dosing: Oral once daily

Why it Matters: Novel mechanism of action, first oral alternative to injectable standards of care as both initial and long-term maintenance treatment, also in development for carcinoid syndrome

Important to Note: Lack of direct head-to-head data vs. competitors, generic availability of alternatives (octreotide, lanreotide)

Estimated Cost: ~$84,000 (based on average pricing of branded therapies)

Pivotal trial data:
The efficacy of paltusotine was evaluated in PATHFNDR-1 and PATHFNDR-2, two Phase 3, randomized, double-blind, placebo-controlled studies in adult patients with acromegaly. The primary endpoint was the proportion of patients who achieved normalized IGF-1 levels, defined as IGF-1 ≤ 1.0 x the upper limit of normal (ULN).

In PATHFNDR-1, 58 patients stabilized on long-acting SRLs (ie, octreotide or lanreotide injections) were randomized to receive paltusotine or placebo for 36 weeks. More patients on paltusotine (83%) than placebo (4%) achieved IGF-1 ≤ 1.0 x ULN (treatment difference: 79%, $p < 0.0001$).

In PATHFNDR-2, 111 medically treatment-naïve patients were randomized to receive paltusotine or placebo for 24 weeks. More paltusotine-treated (56%) than placebo-treated (5%) patients achieved IGF-1 ≤ 1.0 x ULN (treatment difference: 51%, $p < 0.0001$).

Safety:
The most common adverse events with paltusotine use were diarrhea, abdominal pain, and nausea.

Dosing:
In the pivotal trials, paltusotine was administered orally once daily, with doses increased as needed if IGF-1 > 0.9 x ULN.

Paltusotine (continued...)

Competitive environment

If approved, paltusotine will enter an established treatment space as a first-in-class SST2 nonpeptide agonist. Current SRLs for acromegaly include Novartis' Sandostatin® (octreotide; generic available), Ipsen's Somatuline® (lanreotide; generic available), Chiesi's Mycapssa® (octreotide), and Recordati's Signifor® (pasireotide). Paltusotine's key differentiator is its convenient route of administration. The most common pharmacological treatments for acromegaly are Sandostatin, Somatuline, and Signifor, which are injected monthly by healthcare providers, although Somatuline can be self-administered with proper patient training. Mycapssa is an oral twice daily SRL, but it would not directly compete with paltusotine as it is indicated only as switch therapy from octreotide or lanreotide. Paltusotine will be the first oral SRL for both initial treatment and long-term maintenance of acromegaly.

Paltusotine is also being investigated in a Phase 3 study for the treatment of carcinoid syndrome, which is an approved indication of Sandostatin and Somatuline.

In the pivotal studies, paltusotine demonstrated promising outcomes as both initial and switch therapy. However, direct head-to-head studies with alternative SRLs would be needed to establish comparative efficacy. Furthermore, several of the current standards of care are generically available.

For reference, the average WAC for branded drugs used for acromegaly is approximately $84,000 per year.

Tolebrutinib (Brand Name: To be determined)

Manufacturer: Sanofi
Expected FDA decision: September 28, 2025

Therapeutic use

Tolebrutinib is under review for the treatment of non-relapsing secondary progressive multiple sclerosis (nrSPMS) and to slow disability accumulation independent of relapse activity in adult patients.

MS is a chronic, progressive, autoimmune disease of the central nervous system. The disease affects about 1 million people in the U.S. About 85% of patients with MS have the relapsing-remitting (RRMS) subtype. These patients experience clearly defined attacks of new or increasing neurologic symptoms (relapses) followed by periods of partial or complete recovery (remission). Over time, patients will progress to secondary progressive MS (SPMS). This is characterized by steady neurologic function decline and continued disability progression even in the absence of clearly defined relapses (nrSPMS).

Clinical profile

Tolebrutinib is a Bruton's tyrosine kinase (BTK) inhibitor. Tolebrutinib crosses the blood-brain barrier, allowing it to modulate B-lymphocytes and disease-associated microglia within the central nervous system. This is believed to address the underlying pathology of progressive MS by targeting the inflammatory processes that contribute to the disease.

What you need to know:

Proposed Indication: Treatment of nrSPMS and to slow disability accumulation independent of relapse activity in adult patients

Mechanism: BTK inhibitor

Efficacy: 6-month CDP: 22.6% with tolebrutinib vs. 30.7% with placebo

Common AEs: Nasopharyngitis, arthralgia, influenza, hypertension, liver enzyme elevations

Dosing: Oral once daily

Why it Matters: Novel mechanism for treatment of MS, potentially the first approved drug for nrSPMS, first study to demonstrate slowing of disease progression in nrSPMS

Important to Note: Pivotal trials in relapsing MS did not meet primary endpoint, affects an older population (average age in the Phase 3 study was 49 years), class-wide liver safety concern

Estimated Cost: ~$116,000 per year (based on pricing for Ponvory)

Pivotal trial data:
The efficacy of tolebrutinib was evaluated in HERCULES, a phase 3, randomized, double-blind, placebo-controlled study in 1,131 patients with nrSPMS. Patients had to have an absence of clinical relapses in the 24 months before screening. Patients were randomized to tolebrutinib or placebo for up to approximately 48 months. The primary endpoint was 6-month confirmed disability progression (CDP) defined as an increase of ≥ 1.0 point from the baseline Expanded Disability Status Scale (EDSS) score when the baseline score is ≤ 5.0, or an increase of ≥ 0.5 points when the baseline EDSS score was > 5.0.

Sustained CDP progression for at least 6 months occurred in 22.6% of patients with tolebrutinib vs. 30.7% with placebo. This represented a 31% reduction in the risk of disability progression (hazard ratio [HR] 0.69, 95% CI: 0.55, 0.88; p = 0.0026). Further analysis of secondary endpoints demonstrated that the number of patients who experienced confirmed disability improvement increased by nearly two-fold, 10% with tolebrutinib compared to 5% with placebo (HR 1.88, 95% CI: 1.10, 3.21; p = 0.021).

Tolebrutinib (continued...)

Safety:
The most common adverse events with tolebrutinib use were nasopharyngitis, arthralgia, influenza, hypertension, and liver enzyme elevations.

Dosing:
In the pivotal trial, tolebrutinib was administered orally once daily.

Competitive environment

If approved, tolebrutinib would offer a novel, oral treatment for MS and would be the first drug indicated specifically for the nrSPMS population. The current treatment options for MS are generally only effective in patients with relapsing or active forms of MS. The pivotal study (HERCULES) for tolebrutinib was the first to show a significant slowing of disability progression in patients with nrSPMS.

In the more common relapsing MS population, tolebrutinib was also evaluated in two Phase 3 studies (GEMINI trials). These studies did not meet their primary endpoint of reducing the annualized relapse rate, compared to another oral MS drug, Aubagio® (teriflunomide). However, a pooled analysis of the studies did show an improvement in the secondary endpoint of confirmed disability worsening, which provides supportive evidence for the results found in the HERCULES trial.

Due to the failed relapsed MS trials, the likely place in therapy for tolebrutinib will be in patients with nrSPMS, which will limit the overall eligible population for the drug compared to other MS products.

Finally, BTK inhibitors have historically been associated with elevations in liver enzymes, and that pattern held true for tolebrutinib as well. Tolebrutinib will likely require monitoring of hepatic function to prevent liver-associated adverse events.

For reference, the WAC for Ponvory® (ponesimod), the last novel oral MS drug approved by the FDA, is approximately $116,000 per year.

Extended brand pipeline forecast

Optum Rx brand pipeline forecast

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
2025 Possible launch date									
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension; interstitial lung disease	INH	Filed NDA	05/24/2025	Yes	No
ET-400	hydrocortisone	Eton Pharmaceuticals	glucocorticoid	Endocrine disorders	PO	Filed NDA	05/28/2025	No	No
AR-15512	AR-15512	Aerie Pharmaceuticals	TRPM8 agonist	Dry eye disease	OPH	Filed NDA	05/30/2025	No	No
mRNA-1283	COVID-19 vaccine, mRNA	Moderna	messanger RNA	COVID-19 prevention	IM	Filed BLA	05/31/2025	No	No
GMRx2	telmisartan/ amlodipine/ indapamide	George Medicines	angiotensin II receptor blocker/ calcium channel blocker/ diuretic	Hypertension	PO	Filed NDA	06/06/2025	No	No
NP-001	sodium chlorite	Neuvivo	neuroprotective agent	Amyotrophic lateral sclerosis	IV	Filed NDA	06/07/2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
MK-1654	clesrovimab	Merck	RSV targeted monoclonal antibody	Respiratory syncytial virus	IM	Filed BLA	06/10/2025	No	No
Subvenite	lamotrigine	OWP Pharmaceuticals	anticonvulsant	Epilepsy/ bipolar disorder	PO	Filed NDA	2Q2025	No	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	Filed NDA	06/30/2025	Yes	Yes
Irinotecan liposome	irinotecan liposome	CSPC Pharmaceutical	topoisomerase-I inhibitor	Pancreatic cancer	IV	Filed NDA	1H2025	Yes	No
DZD-9008	sunvozertinib	Dizal	EGFR inhibitor	Non-small cell lung cancer	PO	Filed NDA	07/08/2025	Yes	No
REGN-5458	linvoseltamab	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	Filed BLA	07/10/2025	Yes	No
RP-1	vusolimogene oderparepvec	Replimune	oncolytic immunotherapy	Melanoma	Intratumoral	Filed BLA	07/22/2025	Yes	No
LEO-124249	delgocitinib	LEO Pharma	Janus kinase inhibitor	Chronic hand eczema	TOP	Filed NDA	07/23/2025	No	No
PTC-923	sepiapterin	PTC Therapeutics	phenylalanine hydroxylase activator	Phenylketonuria	PO	Filed NDA	07/29/2025	Yes	Yes
REGN-1979	odronextamab	Regeneron	CD20/CD3 monoclonal antibody	Follicular lymphoma/ diffuse large b-cell lymphoma	IV	Filed BLA	07/30/2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
MRNA-1283	Moderna next generation vaccine	Moderna	mRNA	Prevention of COVID-19	IM	Filed BLA	08/2025	No	No
AEB-1102	pegzilarginase	Immedica	enzyme replacement/ arginase-I stimulator	Arginase 1 deficiency	IV	Filed BLA	08/06/2025	Yes	Yes
LNZ-100	aceclidine	Lenz Therapeutics	acetylcholine receptor agonist	Treatment of presbyopia	OPH	Filed NDA	08/08/2025	No	No
BAY-342	elinzanetant	Bayer	neurokinin-1,3 receptor antagonist	Vasomotor symptoms	PO	Filed NDA	08/09/2025	No	No
INS-1007	brensocatib	Insmed	dipeptidyl peptidase 1 inhibitor	Bronchiectasis	PO	Filed NDA	08/12/2025	Yes	No
TNX-102	cyclobenzaprine	Tonix	muscle relaxant	Fibromyalgia	PO	Filed NDA	08/15/2025	No	No
ONC-201	dordaviprone	Chimerix	dopamine receptor antagonist	Brain cancer	PO	Filed NDA	08/18/2025	Yes	Yes
UX-111 (ABO-102)	UX-111	Ultragenyx Pharmaceutical	gene therapy	Sanfilippo syndrome type A	IV	Filed BLA	08/18/2025	Yes	Yes
PTC-743	vatiquinone	PTC Therapeutics	undisclosed	Friedreich's ataxia	PO	Filed NDA	08/19/2025	Yes	Yes
BI-1810631	zongertinib	Boehringer Ingelheim	HER2 exon 20 inhibitor	Non-small cell lung cancer	PO	Filed NDA	08/19/2025	Yes	No
IONIS-PKK-LRx (ISIS-721744)	donidalorsen	Ionis	antisense drug	Hereditary angioedema	SC	Filed NDA	08/21/2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	Filed BLA	08/27/2025	Yes	No
SL-1009	sodium dichloroacetate	Saol Therapeutics	pyruvate dehydrogenase kinase inhibitor	Pyruvate dehydrogenase complex deficiency	PO	Filed NDA	08/27/2025	Yes	Yes
PRGN-2012	zopapogene imadenovec	Precigen	immunotherapy	Respiratory papillomatosis	SC	Filed BLA	08/27/2025	Yes	Yes
PRN-1008	rilzabrutinib	Sanofi	BTK inhibitor	Chronic immune thrombocytopenia	PO	Filed NDA	08/29/2025	Yes	Yes
CAP-1002	deramiocel	Capricor Therapeutics	cellular therapy	Duchenne muscular dystrophy	IV	Filed BLA	08/31/2025	Yes	Yes
MT-1621	deoxythymidine/ deoxycytidine	UCB	deoxynucleoside	Thymidine kinase 2 deficiency	PO	Filed NDA	08/2025	Yes	Yes
Leqembi SC	lecanemab	Eisai/Biogen	beta-amyloid targeted therapy	Alzheimer's disease	SC	Filed BLA	08/31/2025	Yes	No
RSQ-777	bumetanide	Corstasis Therapeutics	diuretic	Edema	Intranasal	Filed NDA	09/14/2025	No	No
SRK-015	apitegromab	Scholar Rock	myostatin activation inhibitor	Spinal muscular atrophy	IV	Filed BLA	09/22/2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
Keytruda SC	pembrolizumab/ hyaluronidase	Merck	programmed death receptor-1-blocking antibody	Various cancers	SC	Filed BLA	09/23/2025	Yes	No
Lydolyte	lidocaine	MEDRx	anesthetic agent	Neuropathic pain	TOP	Filed NDA	09/24/2025	No	No
CRN-00808	paltusotine	Crinetics Pharmaceuticals	somatostatin receptor 2 agonist	Acromegaly	PO	Filed NDA	09/25/2025	Yes	Yes
SAR-442168	tolebrutinib	Sanofi	Bruton's tyrosine kinase inhibitor	Multiple sclerosis	PO	Filed NDA	09/28/2025	Yes	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	Filed BLA	09/2025	Yes	Yes
N-115	sodium pyruvate	EmphyCorp	IL-6 inhibitor	Idiopathic pulmonary fibrosis	Intranasal	Filed NDA	09/2025	Yes	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	Filed NDA	3Q2025	Yes	Yes
BI-1015550	nerandomilast	Boehringer Ingelheim	phosphodiesterase 4b inhibitor	Idiopathic pulmonary fibrosis	PO	Filed NDA	10/01/2025	Yes	Yes
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	Filed NDA	10/07/2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
HY-029	valacyclovir	Hyloris	deoxynucleoside analogue DNA polymerase inhibitor	Herpes Zoster	PO	Filed NDA	10/12/2025	No	No
SYD-101	atropine	Sydnexis	anticholinergic	Myopia	OPH	Filed NDA	10/23/2025	No	No
RGX-121	clemidsogene lanparvovec	Regenxbio	gene therapy	Mucopolysaccharidosis Type II	Intracisternal	Filed BLA	11/09/2025	Yes	Yes
BHV-4157	troriluzole	Biohaven	glutamate modulator	Spinocerebellar ataxia	PO	Filed NDA	11/11/2025	Yes	Yes
OTL-103	OTL-103	Orchard Therapeutics/ Fondazione Telethon	gene therapy	Wiskott-Aldrich syndrome	IV	Filed BLA	11/11/2025	Yes	Yes
TAR-200	gemcitabine	J&J	nucleoside metabolic inhibitor	Bladder cancer	Intravesical	Filed NDA	11/16/2025	Yes	No
ARO-APOC3	plozasiran	Arrowhead Pharmaceuticals	RNAi targeting apolipoprotein C-III	Familial chylomicronemia syndrome	SC	Filed BLA	11/18/2025	Yes	Yes
Molgradex	molgramostim	Savara	granulocyte macrophage-colony stimulating factor	Pulmonary alveolar proteinosis	INH	Filed BLA	11/26/2025	Yes	Yes
KO-539	ziftomenib	Kura	menin inhibitor	Acute myeloid leukemia	PO	Filed NDA	11/30/2025	Yes	No
LIB-003	lerodalcibep	LIB Therapeutics	PCSK9 inhibitor	Hypocholesteremia	SC	Filed BLA	12/12/2025	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
GSK-3511294	depemokimab	GSK	interleukin-5 antagonist	Eosinophilic asthma/ chronic rhinosinusitis	SC	Filed BLA	12/16/2025	Yes	No
CK-274	aficamten	Cytokinetics	cardiac myosin inhibitor	Obstructive hypertrophic cardiomyopathy	PO	Filed NDA	12/26/2025	Yes	Yes
CORT-125134	relacorilant	Corcept Therapeutics	glucocorticoid receptor II antagonist	Cushing's syndrome	PO	Filed NDA	12/30/2025	Yes	Yes
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	CRL	4Q2025	No	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Motion sickness	PO	Filed NDA	4Q2025	No	No
YN-96D1	rivoceranib	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Hepatocellular carcinoma	PO	CRL	4Q2025	Yes	Yes
LY-3484356	imlunestrant	Eli Lilly	selective estrogen receptor degrader	Breast cancer	PO	Filed NDA	4Q2025	Yes	No
SHR-1210	camrelizumab	Elevar Therapeutics	programmed death receptor-1-blocking antibody	Hepatocellular carcinoma	IV	CRL	4Q2025	Yes	Yes
Oral Wegovy (for weight loss)	semaglutide	Novo Nordisk	glucagon-like peptide 1 receptor agonist	Obesity	PO	Filed NDA	4Q2025	No	No
ND-0612H	levodopa/ carbidopa	Mitsubishi Tanabe	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	CRL	2H2025	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
AT-007	govorestat	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	PO	CRL	2H2025	Yes	Yes
SDN-037	difluprednate	Visiox/ Ocuvex Therapeutics	corticosteroid	Ocular inflammation/pain	OPH	InTrial	2H2025	No	No
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	CRL	2H2025	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	2H2025	Yes	Yes
K-127	pyridostigmine	Amneal	cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	2H2025	No	No
CAM-2029	octreotide	Camurus	somatostatin analogue	Acromegaly	SC	CRL	2H2025	Yes	Yes
Rybrevant SC	amivantamab-vmjw/ hyaluronidase	J&J	bispecific EGF receptor-directed and MET receptor-directed antibody	Non-small cell lung cancer	SC	CRL	2H2025	Yes	No
AZD-0914	zoliflodacin	Innoviva	DNA gyrase inhibitor	Gonorrhea	PO	InTrial	Late 2025	No	No
NRX-100	ketamine	NRx Pharmaceuticals	NMDA antagonist	Depression	IV	InTrial	Late 2025	No	No
SEL-212	SVP-rapamycin/ pegsiticase	Selecta Biosciences/ 3SBio	synthetic vaccine particle/ enzyme combination	Gout	IV	InTrial	Late 2025	Yes	No
RP-L201	marnetegragene autotemcel	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	CRL	Late 2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
HER3-DXd	patritumab deruxtecan	Daiichi Sankyo/ Merck	antibody drug conjugate	Non-small cell lung cancer	IV	CRL	Late 2025	Yes	No
DNL-310	tividenofusp alfa	Denali	enzyme replacement therapy	Mucopolysaccharidosis II (Hunter Syndrome)	IV	Filed BLA	Late 2025	Yes	Yes
Zolgensma IT	onasemnogene abeparvovec-xioi	Novartis	gene therapy	Spinal muscular atrophy	Intrathecal	InTrial	Late 2025	Yes	Yes
BT-524	fibrinogen	Biotest	plasma fibrinogen concentrate	Fibrinogen deficiency	IV	Filed BLA	Late 2025	Yes	No
2026 Possible launch date									
VTI-001 (Brimochol PF)	brimonidine/ carbachol	Tenpoint Therapeutics	cholinergic muscarinic receptor agonist/ parasympathomimetic	Presbyopia	OPH	Filed NDA	02/08/2026	No	No
VHX-896	milsaperidone	Vanda Pharmaceuticals	atypical antipsychotic	Schizophrenia/ bipolar disorder	PO	Filed NDA	02/21/2026	No	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	1Q2026	No	No
NRX-101	d-cycloserine/ lurasidone	NRx Pharmaceuticals	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	1Q2026	No	No
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	1Q2026	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
ANX-005	ANX-005	Annexon	C1q inhibitor	Guillain-Barré syndrome	IV	InTrial	1Q2026	Yes	Yes
AQST-109	epinephrine	Aquestive Therapeutics	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	PO	Filed NDA	1Q2026	No	No
INO-3107	INO-3107	Inovio Pharmaceuticals	immunotherapy	Recurrent respiratory papillomatosis	IM	InTrial	1Q2026	Yes	Yes
TransCon CNP	navepegritide	Ascendis Pharma	C-type natriuretic peptide	Achondroplasia	SC	Filed NDA	03/31/2026	Yes	Yes
VIS-649	sibeprenlimab	Otsuka	cytokine APRIL inhibitor	IgA nephropathy	SC	Filed BLA	03/31/2026	Yes	No
cytisine	cytisine	Achieve Life Sciences	nicotinic acetylcholine receptor antagonist	Smoking cessation	PO	InTrial	2Q2026	No	No
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OPH	CRL	1H2026	No	No
DISC-1459	bitopertin	Disc Medicine	glycine transporter 1 inhibitor	Erythropoietic protoporphyria	PO	InTrial	1H2026	No	Yes
AXS-14	esreboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	Filed NDA	1H2026	No	No
CNM-Au8	CNM-Au8	Clene	gold nanocrystal	Amyotrophic lateral sclerosis	PO	InTrial	1H2026	Yes	Yes
MSP-2017	etripamil	Milestone	calcium channel blocker	Arrhythmia	Intranasal	CRL	1H2026	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
LY-3209590	insulin efsitora alfa	Eli Lilly	ultra-long acting basal insulin	Diabetes mellitus	SC	InTrial	1H2026	No	No
LOU-064	remibrutinib	Novartis	Bruton's tyrosine kinase inhibitor	Chronic spontaneous urticaria	PO	InTrial	1H2026	Yes	No
GSK-2330672	linerixibat	GSK	ileal bile acid transfer inhibitor	Primary biliary cholangitis	PO	InTrial	1H2026	Yes	Yes
ANB-019	imsidolimab	AnaptysBio/ Vanda Pharmaceuticals	interleukin-36 receptor antagonist	Generalized pustular psoriasis	IV	InTrial	1H2026	Yes	Yes
Mim8	Mim8	Novo Nordisk	Factor VIII mimetic bispecific antibody	Hemophilia A	SC	InTrial	1H2026	Yes	Yes
DTX-401	pariglasgene brecaparvovec	Ultragenyx Pharmaceutical	gene therapy	Glycogen storage disease type Ia	IV	InTrial	1H2026	Yes	Yes
Donesta	estetrol	Mithra Pharmaceuticals	estrogen	Vasomotor symptoms	PO	InTrial	1H2026	No	No
AGEN-1181	botensilimab	Agenus	anti-CTLA-4 antibody	Colorectal cancer	IV	InTrial	1H2026	Yes	No
XS-003	nilotinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	InTrial	1H2026	Yes	No
Sarclisa SC	isatuximab-irfc	Sanofi	CD38-directed cytolytic antibody	Multiple myeloma	SC	InTrial	1H2026	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
MCO-010	sonpiretigene isteparvovec	Nanoscope Therapeutics	gene therapy	Retinitis pigmentosa	Intravitreal	InTrial	1H2026	Yes	Yes
HLX-10	serplulimab	Henlius	anti-PD-1	Small cell lung cancer	IV	InTrial	1H2026	Yes	Yes
Aversa Fentanyl	fentanyl	Nutriband	opioid agonist	Pain	TOP	InTrial	1H2026	No	No
SPR-994	tebipenem	Spero Therapeutics/ GSK	carbapenem	Complicated urinary tract infections	PO	CRL	Mid-2026	No	No
IdeS (immunoglobulin G-degrading enzyme of Streptococcus pyogenes)	imlifidase	Hansa Medical	bacterial enzyme	Kidney transplant	IV	InTrial	Mid-2026	Yes	Yes
SRP-9003	bidridistrogene xeboparvovec	Sarepta Therapeutics	gene therapy	Limb-girdle muscular dystrophy	IV	InTrial	Mid-2026	Yes	Yes
CTI-1601	nomlabofusp	Larimar Therapeutics	recombinant fusion protein	Friedreich's ataxia	SC	InTrial	Mid-2026	Yes	Yes
Trappsol Cyclo	beta-cyclodextrin	Cyclo Therapeutics	cyclodextrin	Niemann-Pick disease Type C	IV	InTrial	Mid-2026	Yes	Yes
zidesamtinib	zidesamtinib	Nuvalent	ROS1 inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2026	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
CTX-1301	dexmethylphenidate	Cingulate	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	3Q2026	TBD	No
mRNA-1083	influenza and COVID-19 vaccine	Moderna	mRNA	Prevention of influenza and COVID-19	IM	InTrial	3Q2026	No	No
SD-101	allantoin	Paradigm Therapeutics	antioxidant	Epidermolysis bullosa	TOP	InTrial	4Q2026	Yes	Yes
ST-920	isaralgagene civaparvovec	Sangamo Therapeutics	gene therapy	Fabry disease	IV	InTrial	4Q2026	Yes	Yes
BLI-5100	tegoprazan	Sebela Pharmaceuticals/ Braintree Laboratories	potassium competitive acid pump antagonist	Gastroesophageal reflux disease	PO	InTrial	4Q2026	No	No
MR-107A-02	meloxicam	Viatris	non-steroidal anti-inflammatory drug	Pain	PO	InTrial	4Q2026	No	No
PH-94B	fasedienol	VistaGen Therapeutics	vomerophine	Social anxiety disorder	Intranasal	InTrial	2H2026	No	No
TRN-228	cebranopadol	Tris Pharma	opioid-receptor-like-1 agonist	Pain	PO	InTrial	2H2026	No	No
AXS-12	reboxetine	Axsome Therapeutics	norepinephrine reuptake inhibitor	Narcolepsy	PO	InTrial	2H2026	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
obicetrapib	obicetrapib	NewAmsterdam Pharma	selective cholesteryl ester transfer protein inhibitor	Hypercholesterolemia	PO	InTrial	2H2026	No	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2H2026	Yes	No
BLU-5937	camlipixant	GSK	P2X3 receptor antagonist	Refractory chronic cough	PO	InTrial	2H2026	No	No
atacicept	atacicept	Vera	transmembrane activator and CAML interactor receptor-immunoglobulin	IgA nephropathy	SC	InTrial	2H2026	Yes	No
LNA-043	LNA-043	Novartis	chondrogenesis inducer	Osteoarthritis	Intraarticular	InTrial	2H2026	Yes	No
Revascor	rexlemestrocel-L	Mesoblast	allogeneic autologous mesenchymal precursor cell	Heart failure	IV	InTrial	2H2026	Yes	Yes
SB-525	giroctocogene fitelparvovec	Sangamo Therapeutics	gene therapy	Hemophilia A	IV	InTrial	2H2026	Yes	Yes
SAR-440340	itepekimab	Sanofi/ Regeneron	anti-interleukin-33 monoclonal antibody	Chronic obstructive pulmonary disease	SC	InTrial	2H2026	Yes	No
XEN-1101	XEN-1101	Xenon Pharmaceuticals	Kv7 potassium channel opener	Focal epilepsy	PO	InTrial	2H2026	TBD	No
VRDN-001	veligrotug	Viridian Therapeutics	insulin-like growth factor-1 receptor inhibitor	Thyroid eye disease	IV	InTrial	2H2026	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
REGN-3767	fianlimab	Regeneron	anti lymphocyte-activation gene 3 monoclonal antibody	Melanoma	IV	InTrial	2H2026	Yes	No
PTG-300	rusfertide	Protagonist Therapeutics	hepcidin mimetic peptide	Polycythemia vera	SC	InTrial	2H2026	Yes	Yes
MTX-005	MTX-005	Memo Therapeutics	monoclonal antibody	BKV infection	IV	InTrial	2H2026	TBD	No
LYN-005	risperidone	Lyndra Therapeutics	atypical antipsychotic	Schizophrenia	PO	InTrial	2H2026	No	No
JNJ-2113	icotrokinra	Janssen/ Protagonist Therapeutics	interleukin-23 receptor antagonist	Plaque psoriasis	PO	InTrial	2H2026	Yes	No
NMRA-140	navacaprant	Neumora Therapeutics	kappa opioid receptor antagonist	Major depressive disorder	PO	InTrial	2H2026	No	No
TSR-022	cobolimab	GSK	anti-TIM-3 antibody	Non-small cell lung cancer	IV	InTrial	2H2026	Yes	No
INCB-54707	povorcitinib	Incyte	Janus kinase inhibitor	Hidradenitis suppurativa	PO	InTrial	2H2026	Yes	No
TV-44749	olanzapine	Teva	atypical antipsychotic	Schizophrenia	SC	InTrial	2H2026	No	No
BPN-14770	zatolmilast	Shionogi	phosphodiesterase 4 inhibitor	Fragile X syndrome	PO	InTrial	2H2026	Yes	Yes
RG-6149	astegolimab	Roche	interleukin-33 antagonist	Chronic obstructive pulmonary disease	SC	InTrial	2H2026	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
TAK-861	oveporexton	Takeda	selective orexin receptor agonist	Narcolepsy	PO	InTrial	2H2026	No	No
cilostazol extended-release	cilostazol	Genovate Biotechnology	phosphodiesterase III inhibitor	Peripheral arterial disease	PO	InTrial	2H2026	No	No
tavapadon	tavapadon	AbbVie	dopamine partial agonist	Parkinson's disease	PO	InTrial	2H2026	No	No
RP-A501	RP-A501	Rocket Pharmaceuticals	gene therapy	Danon disease	IV	InTrial	2H2026	Yes	Yes
OST-HER2	OST-HER2	OS Therapies	immunotherapy	Osteosarcoma	IV	InTrial	2H2026	Yes	Yes
CGT-9486	bezuclastinib	Cogent Biosciences	KIT inhibitor	Mastocytosis	PO	InTrial	2H2026	Yes	No
AGEN-2034	balstilimab	Agenus	PD-1 antagonist	Colorectal cancer	IV	InTrial	2026	Yes	No
Hernicore (SI-6603)	condoliase	Seikagaku	glycosaminoglycan-degrading enzyme	Pain	Intrathecal	CRL	2026	Yes	No
EB-1020	centanafadine	Otsuka	norepinephrine, dopamine and serotonin reuptake inhibitor	Attention deficit hyperactivity disorder	PO	InTrial	2026	No	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	CRL	2026	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
RP-5063	brilaroxazine	Reviva Pharmaceuticals	dopamine/serotonin parital agonist	Schizophrenia/ schizoaffective disorder	PO	InTrial	2026	No	No
ASP-1929 (RM-1929)	ASP-1929	Rakuten	EGFR inhibitor	Head and neck squamous cell carcinoma	IV	InTrial	2026	Yes	No
PDP-716	brimonidine	Visiox Pharma/ Ocuvex Therapeutics	alpha-2 agonist	Glaucoma	OPH	InTrial	2026	No	No
SLS-001 (WT-1)	galinpepimut-S	Sellas Life Sciences Group	vaccine	Acute myeloid leukemia	SC	InTrial	2026	Yes	Yes
Mino-Lok	minocycline-EDTA-ETOH	Citrus	tetracyclines	Bacterial infection	Intracatheter	InTrial	2026	No	No
REGN-2477	garetosmab	Regeneron	Activin A antibody	Fibrodysplasia ossificans progressiva	IV/SC	InTrial	2026	Yes	Yes
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer/ hepatocellular carcinoma	IV	InTrial	2026	Yes	No
REGN-4461	mibavademab	Regeneron	leptin receptor agonist	Generalized lipodystrophy	IV	InTrial	2026	Yes	No
CPI-0610	pelabresib	MorphoSys	BET inhibitor	Myelofibrosis	PO	InTrial	2026	Yes	Yes
EB-613	teriparatide	Entera Bio	parathyroid hormone	Osteoporosis	PO	InTrial	2026	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	Implant	InTrial	2026	No	No
CT-053	zevorcabtagene autoleucel	CARsgen Therapeutics	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Multiple myeloma	IV	InTrial	2026	Yes	Yes
BBP-305	encaleret	BridgeBio	Ca sensing receptor antagonist	Autosomal dominant hypocalcemia type 1	PO	InTrial	2026	Yes	Yes
CART-ddBCMA	anitocabtagene autoleucel	Arcellx	CAR T cell therapy	Multiple myeloma	IV	InTrial	2026	Yes	No
mRNA-1010	mRNA-1010	Moderna	vaccine	Influenza	IM	InTrial	2026	No	No
FP-1304	bexmarilimab	Faron	anti-Clever-1 antibody	Myelodysplastic syndrome	IV	InTrial	2026	Yes	No
QRX-003	QRX-003	Quoin Pharmaceuticals	serine protease inhibitor	Netherton syndromes	TOP	InTrial	2026	TBD	No
IO102-IO103	IO102-IO103	IO Biotech	vaccine	Melanoma	SC	InTrial	2026	Yes	No
ION-373	zilganersen	Ionis Pharmaceuticals	antisense oligonucleotide	Alexander disease	Intrathecal	InTrial	2026	Yes	Yes
CG-0070	cretostimogene grenadenorepvec	CG Oncology	oncolytic immunotherapy	Bladder cancer	Intravesical	InTrial	2026	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected Launch Date	Specialty	Orphan Drug Status
ONO-4059	tirabrutinib	Ono	Bruton's tyrosine kinase inhibitor	Primary central nervous system lymphoma	PO	InTrial	2026	Yes	Yes
TQJ-230 (ISIS-681257)	pelacarsen	Novartis	antisense oligonucleotide targeting Lp(a)	Cardiovascular disease	SC	InTrial	Late 2026	TBD	No
PF-07940370	inclacumab	Pfizer	P-selectin inhibitor	Sickle cell disease	IV	InTrial	Late 2026	Yes	Yes
AMG-451	rocatinlimab	Amgen/ Kyowa Kirin	anti-OX40 monoclonal antibody	Atopic dermatitis	SC	InTrial	Late 2026	Yes	No
LY-3502970	orforglipron	Eli Lilly	glucagon-like peptide-1 agonist	Diabetes/ obesity	PO	InTrial	Late 2026	No	No
CHR-3996	nanatinostat	Viracta Therapeutics	histone deacetylase inhibitor	Peripheral T-cell lymphoma	PO	InTrial	Late 2026	Yes	No

IM = intramuscular, INH = inhalation, INJ = injection, IUD = intrauterine device, IV = intravenous, OPH = ophthalmic, PO = oral, SC = subcutaneous, TOP = topical

Key pending indication forecast

Optum Rx key pending indication forecast

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
2025 Possible launch date								
MenQuadfi	meningococcal groups A, C, Y, W	Sanofi	vaccine	Revised	Active immunization for the prevention of invasive meningococcal disease caused by Neisseria meningitidis serogroups A, C, W, and Y in children aged 6 weeks to 23 months	IM	Filed sBLA	05/23/2025
Ixchiq	chikungunya vaccine	Valneva	vaccine	Revised	Prevention of disease caused by chikungunya virus (CHIKV) in adolescents aged 12 to 17 years who are at increased risk of exposure to CHIKV	IM	Filed sBLA	05/26/2025
Sunlenca	lenacapavir	Gilead	HIV-1 capsid inhibitor	New	In at-risk patients for pre-exposure prophylaxis (PrEP) to reduce the risk of sexually acquired HIV-1 infection	SC	Filed sNDA	06/19/2025
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	New	Treatment of bullous pemphigoid	SC	Filed sBLA	06/20/2025
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	New	Treatment of patients with resectable locally advanced head and neck squamous cell carcinoma as neoadjuvant	IV	Filed sBLA	06/23/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
					treatment, then continued as adjuvant treatment in combination with standard of care radiotherapy with or without cisplatin and then as a single agent			
Gamifant	emapalumab-lzsg	Sobi	interferon gamma blocking antibody	Revised	Treatment of adult and pediatric patients with hemophagocytic lymphohistiocytosis/macrophage activation syndrome (MAS) in Still's disease with an inadequate response or intolerance to glucocorticoids, or with recurrent MAS	IV	Filed sBLA	06/27/2025
Darzalex Faspro	daratumumab/ hyaluronidase-fihj	J&J	humanized anti-CD38 monoclonal antibody	New	In combination with bortezomib, lenalidomide and dexamethasone for the treatment of adult patients with newly diagnosed multiple myeloma for whom autologous stem cell transplant (ASCT) is deferred or who are ineligible for ASCT	SC	Filed sBLA	2Q2025
Darzalex Faspro	daratumumab/ hyaluronidase-fihj	J&J	humanized anti-CD38 monoclonal antibody	New	Treatment of adult patients with high-risk smouldering multiple myeloma	SC	Filed sBLA	2Q2025
mRESVIA	respiratory syncytial virus vaccine	Moderna	vaccine	Revised	Prevention of RSV infection in high-risk adults aged 18 to 59	IM	Filed sBLA	07/13/2025
Kerendia	finerenone	Bayer	non-steroidal mineralocorticoid receptor antagonist	New	Treatment of adult patients with heart failure with a left ventricular ejection fraction of ≥40%, i.e.,	PO	Filed sNDA	07/17/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
					mildly reduced LVEF (HFmrEF) or preserved LVEF (HFpEF)			
Columvi	glofitamab-gxbm	Genentech	bispecific CD20-directed CD3 T-cell engager	New	In combination with gemcitabine and oxaliplatin for the treatment of people with relapsed or refractory diffuse large B-cell lymphoma who have received at least one prior line of therapy and are not candidates for autologous stem cell transplant	IV	Filed sBLA	07/20/2025
Nubeqa	darolutamide	Bayer	androgen receptor inhibitor	Revised	In combination with androgen deprivation therapy in patients with metastatic hormone-sensitive prostate cancer	PO	Filed sNDA	07/21/2025
Blenrep	belantamab mafodotin-blmf	GSK	BCMA-directed antibody and microtubule inhibitor conjugate	New	In combinations with bortezomib plus dexamethasone or pomalidomide plus dexamethasone for the treatment of patients with multiple myeloma who have received at least one prior line of therapy	IV	Filed sBLA	07/23/2025
Doptelet	avatrombopag	Sobi	thrombopoietin receptor agonist	Revised	Treatment of thrombocytopenia in pediatric patients one year and older with persistent or chronic immune thrombocytopenia who have had an insufficient response to a prior therapy	PO	Filed sBLA	07/24/2025
Skytrofa	lonapegsomatropin-tcgd	Ascendis Pharma	growth hormone	Revised	Treatment of adults with growth hormone deficiency	SC	Filed sBLA	07/27/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
Empaveli	pegcetacoplan	Apellis	complement inhibitor	New	Treatment of C3 glomerulopathy and C3 glomerulonephritis	SC	Filed sBLA	07/28/2025
Wegovy	semaglutide	Novo Nordisk	glucagon-like peptide 1 receptor agonist	New	Treatment of metabolic dysfunction-associated steatohepatitis	PO	Filed sNDA	08/19/2025
Eylea HD	aflibercept	Regeneron	vascular endothelial growth factor inhibitor	New	Treatment of retinal vein occlusion	Intravitreal	Filed sBLA	08/19/2025
Pyrukynd	mitapivat	Agios Pharmaceuticals	pyruvate kinase activator	New	Treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia	PO	Filed sNDA	09/07/2025
Orladeyo	berotralstat	BioCryst Pharmaceuticals	plasma kallikrein inhibitor	Revised	Prophylaxis to prevent attacks of hereditary angioedema in adults and pediatric patients 2 years and older	PO	Filed sNDA	09/12/2025
Elyxyb	celecoxib	Scilex	NSAID	New	Treatment of acute pain in adult patients	PO	Filed sNDA	09/21/2025
Wegovy	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	Treatment of adults with heart failure with preserved ejection fraction and obesity	SC	Filed sNDA	3Q2025
Rexulti	brexpiprazole	Otsuka/ Lundbeck	atypical antipsychotic	New	In combination with sertraline for the treatment of post-traumatic stress disorder in adults	PO	Filed sNDA	3Q2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
Opzelura	ruxolitinib	Incyte	Janus kinase inhibitor	Revised	Treatment of pediatric atopic dermatitis	TOP	Filed sNDA	3Q2025
Tremfya	guselkumab	Janssen	interleukin-23 inhibitor	New	Treatment of pediatric juvenile psoriatic arthritis	SC	Filed sBLA	10/02/2025
Tremfya	guselkumab	Janssen	interleukin-23 inhibitor	Revised	Treatment of patients ages 6 years and older with moderate-to-severe plaque psoriasis	SC	Filed sBLA	10/02/2025
Caplyta	lumateperone	Intra-Cellular Therapies	atypical antipsychotic	New	Adjunctive therapy to antidepressants for the treatment of major depressive disorder	PO	Filed sNDA	10/03/2025
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Revised	Treatment of mild-to-moderate atopic dermatitis in patients 2 years and older	TOP	Filed sNDA	10/13/2025
Simponi	golimumab	Janssen	tumor necrosis factor blocker	Revised	Treatment of children two years and older with moderately to severely active ulcerative colitis	IV	Filed sBLA	10/16/2025
Tezspire	tezepelumab-ekko	Amgen/ AstraZeneca	thymic stromal lymphopoietin blocker	New	Treatment of chronic rhinosinusitis with nasal polyps	SC	Filed sBLA	10/19/2025
Uzedy	risperidone	Teva Pharmaceuticals	atypical antipsychotic	New	Treatment of bipolar I disorder	SC	Filed sNDA	10/25/2025
Gazyva	obinutuzumab	Roche	CD20-directed cytolytic antibody	New	Treatment of lupus nephritis	IV	Filed sBLA	10/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised/Removed Indication	Route	FDA Status	Projected Approval Date
Rybelsus	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	To reduce the risk of major adverse cardiovascular events (cardiovascular death, non-fatal heart attack or non-fatal stroke) in people with type 2 diabetes and established cardiovascular disease and/or chronic kidney disease	PO	Filed sNDA	10/2025
Revuforj	revumenib	Syndax Pharmaceuticals	menin inhibitor	New	Treatment of relapsed/refractory mutant NPM1 acute myeloid leukemia	PO	Filed sNDA	10/2025
Uplizna	inebilizumab-cdon	Amgen	CD19-directed cytolytic antibody	New	Treatment of generalized myasthenia gravis	IV	Filed sBLA	12/14/2025
Ozempic	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	Adjunct to standard of care in people living with type 2 diabetes and peripheral arterial disease with intermittent claudication	SC	Filed sNDA	4Q2025
2026 Possible launch date								
Filspari	sparsentan	Travere Therapeutics	endothelin/angiotensin II receptor antagonist	New	Treatment of focal segmental glomerulosclerosis	PO	Filed sNDA	01/13/2026
Ajovy	fremanezumab	Teva	calcitonin gene-related peptide antagonist	Revised	Prevention of episodic migraine in children and adolescent patients aged 6-17 years who weigh 45 kilograms (99 pounds) or more	SC	Filed sNDA	02/07/2026

Extended generic and biosimilar pipline forecast

Optum Rx generic and biosimilar pipeline forecast

(Bolded fields are Biosimilar products)

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
2025 Possible launch date					
TYSABRI	natalizumab	Biogen	Multiple Sclerosis; Crohn's Disease	Intravenous	2025
ISENTRESS	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	2025
NATAZIA	estradiol valerate/dienogest	Bayer	Prevention of Pregnancy; Menorrhagia	Oral	2025
GATTEX	teduglutide recombinant	Takeda	Short Bowel Syndrome	Subcutaneous	2025
PHOSLYRA	calcium acetate	Fresenius	Phosphate Binder	Oral	2025
FINACEA	azelaic acid	LEO Pharma	Rosacea	External	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Human Immunodeficiency Virus-1 Infection	Oral	2025
VUITY	pilocarpine	AbbVie	Presbyopia	Ophthalmic	2025
HUMALOG	insulin lispro	Eli Lilly	Type 1 and 2 Diabetes Mellitus	Subcutaneous	2Q-2025
HORIZANT	gabapentin enacarbil	Arbor	Restless Legs Syndrome; Postherpetic Neuralgia	Oral	2Q-2025
TIROSINT-SOL	levothyroxine	IBSA Institut Biochemique	Hypothyroidism; Thyrotropin-Dependent Thyroid Cancer	Oral	05-2025
FYCOMPA	perampanel	Catalyst	Partial-Onset Seizures; Primary Generalized Tonic-Clonic Seizures	Oral	05-2025
PROLIA	denosumab	Amgen	Postmenopausal Osteoporosis; Bone Loss in Men and Women at Risk of Fracture	Subcutaneous	05-2025
XGEVA	denosumab	Amgen	Prevention of Fractures in Bone Malignancies and Multiple Myeloma; Giant Cell Tumor in Bone; Hypercalcemia	Subcutaneous	05-2025
ZTLIDO	lidocaine	Sorrento	Pain Associated with Post-Herpetic Neuralgia	External	2H-2025
TASIGNA	nilotinib	Novartis	Philadelphia Chromosome-Positive Chronic Myeloid Leukemia	Oral	2H-2025
ENTRESTO	sacubitril/valsartan	Novartis	Heart Failure	Oral	3Q-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
GIAZO	balsalazide disodium	Bausch Health	Ulcerative Colitis in Male Patients	Oral	07-2025
NOVOLOG	insulin aspart	Novo Nordisk	Type 1 and 2 Diabetes Mellitus	Subcutaneous	07-2025
RAVICTI	glycerol phenylbutyrate	Amgen	Urea Cycle Disorders	Oral	07-2025
RYANODEX	dantrolene	Eagle Pharmaceuticals	Malignant Hyperthermia	Intravenous	07-2025
RYTARY	carbidopa/levodopa	Amneal	Parkinson's Disease	Oral	07-2025
DIACOMIT	stiripentol	Biocodex	Dravet Syndrome	Oral	08-2025
ADZENYS XR-ODT	amphetamine polistirex	Neos Therapeutics	Attention Deficit Hyperactivity Disorder	Oral	09-2025
SIMPONI	golimumab	Janssen	Ankylosing Spondylitis; Psoriatic Arthritis; Rheumatoid Arthritis; Ulcerative Colitis	Subcutaneous	4Q-2025
SIMPONI ARIA	golimumab	Janssen	Rheumatoid Arthritis; Psoriatic Arthritis; Ankylosing Spondylitis; Juvenile Idiopathic Arthritis	Intravenous	4Q-2025
BOSULIF	bosutinib	Pfizer	Chronic Myelogenous Leukemia	Oral	4Q-2025
2026 Possible launch date					
SIMBRINZA	brimonidine/brinzolamide	Alcon	Reduction of Elevated Intraocular Pressure in Patients with Open-Angle Glaucoma or Ocular Hypertension	Ophthalmic	2026
XOLAIR	omalizumab	Genentech/Novartis	Asthma; Idiopathic Urticaria; Nasal Polyps; IgE-Mediated Food Allergy	Subcutaneous	2026
BRYHALI	halobetasol	Bausch Health	Plaque Psoriasis	External	2026
MAVENCLAD	cladribine	Serono	Multiple Sclerosis	Oral	2026
POMALYST	pomalidomide	Celgene	Multiple Myeloma; Kaposi Sarcoma	Oral	1Q-2026
YONSA	abiraterone	Sun	Prostate Cancer	Oral	01-2026
GELNIQUE	oxybutynin	Allergan	Overactive Bladder	External	01-2026
QBRELIS	lisinopril	Silvergate	Hypertension; Heart Failure; Acute Myocardial Infarction	Oral	01-2026
VELPHORO	sucroferric oxyhydroxide	Vifor Fresenius Medical Care Renal Pharma (VFMCRP)	Hyperphosphatemia In Patients with Chronic Kidney Disease on Dialysis	Oral	01-2026
BYVALSON	nebivolol/valsartan	AbbVie	Hypertension	Oral	01-2026
JEVTANA	cabazitaxel	Sanofi	Hormone-Refractory Metastatic Prostate Cancer	Intravenous	01-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
EDARBI	azilsartan kamedoxomil	Arbor	Hypertension	Oral	01-2026
SERNIVO	betamethasone dipropionate	Encore Dermatology	Plaque Psoriasis	External	01-2026
ELLA	ulipristal	Afaxys/Perrigo	Emergency Contraception	Oral	01-2026
TYVASO	treprostinil	United Therapeutics	Pulmonary Arterial Hypertension; Pulmonary Hypertension with Interstitial Lung Disease	Inhalation	01-2026
IZBA	travoprost	Alcon	Open-Angle Glaucoma; Ocular Hypertension	Ophthalmic	01-2026
IMPOYZ	clobetasol propionate	Encore Dermatology/Dr. Reddy's	Psoriasis	External	01-2026
BRIVIACT	brivaracetam	UCB	Epilepsy	Oral; intravenous	02-2026
SAVELLA	milnacipran	AbbVie	Fibromyalgia	Oral	03-2026
XELJANZ XR	tofacitinib	Pfizer	Rheumatoid Arthritis; Psoriatic Arthritis; Ulcerative Colitis; Ankylosing Spondylitis	Oral	2Q-2026
XELJANZ	tofacitinib	Pfizer	Rheumatoid Arthritis; Ulcerative Colitis; Psoriatic Arthritis; Juvenile Idiopathic Arthritis; Ankylosing Spondylitis	Oral	2Q-2026
OFEV	nintedanib	Boehringer Ingelheim	Idiopathic Pulmonary Fibrosis; Systemic Sclerosis-Associated Interstitial Lung Disease (ILD); Chronic Fibrosing ILD	Oral	04-2026
QTERN	dapagliflozin/saxagliptin	AstraZeneca	Type 2 Diabetes Mellitus	Oral	04-2026
NULOJIX	belatacept	Bristol-Myers Squibb	Prophylaxis of Organ Rejection in Kidney Transplant	Intravenous	04-2026
JANUVIA	sitagliptan	Merck	Type 2 Diabetes Mellitus	Oral	05-2026
JANUMET	sitagliptan/metformin	Merck	Type 2 Diabetes Mellitus	Oral	05-2026
EVOMELA	melphalan	Acrotech/Aurobindo	Multiple Myeloma; Conditioning for Stem Cell Transplant	Intravenous	06-2026
CERDELGA	eliglustat	Sanofi/Genzyme	Gaucher Disease Type 1	Oral	06-2026
SUPPRELIN LA	histrelin	Endo	Central Precocious Puberty	Subcutaneous	06-2026
COTEMPLA XR-ODT	methylphenidate	Neos Therapeutics	Attention Deficit Hyperactivity Disorder	Oral	07-2026
INJECTAFER	ferric carboxymaltose	American Regent/CSL Limited	Iron Deficiency Anemia	Intravenous	07-2026
JANUMET XR	sitagliptin/metformin	Merck	Type 2 Diabetes Mellitus	Oral	07-2026
NUEDEXTA	dextromethorphan/quinidine sulfate	Avanir	Pseudobulbar Affect	Oral	07-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
COMETRIQ	cabozantinib (S)-malate	Exelixis	Medullary Thyroid Cancer	Oral	08-2026
ILARIS	canakinumab	Novartis	Cryopyrin-Associated Periodic Syndromes; Familial Cold Autoinflammatory Syndrome; Muckle-Wells Syndrome; Tumor Necrosis Factor Receptor Associated Periodic Syndrome; Hyperimmunoglobulin D Syndrome/Mevalonate Kinase Deficiency; Familial Mediterranean Fever; Still's Disease; Gout Flares	Subcutaneous	4Q-2026
ADEMPAS	riociguat	Bayer	Pulmonary Arterial Hypertension; Chronic Thromboembolic Pulmonary Hypertension	Oral	4Q-2026
UPTRAVI	selexipag	Janssen	Pulmonary Arterial Hypertension	Oral	10-2026
VEREGEN	sinecatechins	Sandoz	External Genital and Perianal Warts	External	10-2026
HEMADY	dexamethasone	Acrotech Biopharma	Multiple Myeloma	Oral	10-2026
CYRAMZA	ramucirumab	Eli Lilly	Gastric Cancer; Gastroesophageal Cancer; Metastatic Gastric Cancer; Non-Small Cell Lung Cancer	Intravenous	10-2026
ADASUVE	loxapine	Alexza	Agitation Associated with Schizophrenia or Bipolar Disorder	Inhalation	10-2026
SYNRIBO	omacetaxine mepesuccinate	Teva	Chronic Myelogenous Leukemia	Subcutaneous	10-2026
AVYCAZ	ceftazidime/avibactam	AbbVie	Intra-Abdominal Infections; Urinary Tract Infections, including Pyelonephritis; Pneumonia; Bacterial Pneumonia	Intravenous	11-2026
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Major Depressive Disorder	Oral	12-2026
2027 Possible launch date					
FIRMAGON	degarelix	Ferring	Prostate Cancer	Subcutaneous	2027
KYPROLIS	carfilzomib	Amgen	Multiple Myeloma	Intravenous	2027
ORENCIA	abatacept	Bristol-Myers Squibb	Rheumatoid Arthritis; Juvenile Idiopathic Arthritis; Psoriatic Arthritis; Graft Vs. Host Disease	Intravenous; subcutaneous	2027
SAXENDA	liraglutide	Novo Nordisk	Chronic Weight Management	Subcutaneous	2027
IBRANCE	palbociclib	Pfizer	Breast Cancer	Oral	1Q-2027
BONJESTA	doxylamine/pyridoxine	Duchesnay	Nausea and Vomiting Associated with Pregnancy	Oral	01-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
DIFICID	fidaxomicin	Merck	Treatment of Clostridium difficile-Associated Diarrhea	Oral	01-2027
OSPHENA	ospemifene	Duchesnay	Menopause Symptoms; Dyspareunia	Oral	01-2027
BELEODAQ	belinostat	Acrotech/Aurobindo	Relapsed or Refractory Peripheral T-cell Lymphoma	Intravenous	01-2027
VIBATIV	telavancin	Cumberland	Infections	Intravenous	01-2027
CUBICIN RF	daptomycin	Merck	Complicated Skin and Skin Structure Infections; Staphylococcus aureus Bloodstream Infections	Intravenous	01-2027
PERJETA	pertuzumab	Genentech	HER-2 Positive Breast Cancer	Intravenous	01-2027
ENVARSUS XR	tacrolimus	Veloxis	Prophylaxis of Organ Rejection in Kidney Transplant Patients	Oral	01-2027
RYDAPT	midostaurin	Novartis	Acute Myeloid Leukemia; Systemic Mastocytosis; Mast Cell Leukemia	Oral	01-2027
JUBLIA	efinaconazole	Bausch Health	Onychomycosis of the Toenail	External	01-2027
VALTOCO	diazepam	Neurelis	Epilepsy	Intranasal	01-2027
VIVITROL	naltrexone	Alkermes	Alcohol and/or Opioid Dependence	Intramuscular	01-2027
BELBUCA	buprenorphine	BioDelivery Sciences International	Severe Pain	Oral	01-2027
NATPARA	parathyroid hormone 1-84	Takeda	Hypoparathyroidism	Subcutaneous	01-2027
SUBSYS	fentanyl	BTcP Pharma	Breakthrough Pain in Cancer Patients	Oral	01-2027
NEVANAC	nepafenac	Harrow Health	Pain and Inflammation Associated with Cataract Surgery	Ophthalmic	01-2027
ALTABAX	retapamulin	Aqua Pharmaceuticals/Almirall	Impetigo	External	02-2027
BYDUREON	exenatide	AstraZeneca	Type 2 Diabetes Mellitus	Subcutaneous	02-2027
VITEKTA	elvitegravir	Gilead	Human Immunodeficiency Virus-1 Infection	Oral	02-2027
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Treatment of Moderate to Severe Vasomotor Symptoms Associated with Menopause; Prevention of Postmenopausal Osteoporosis	Oral	03-2027
DYANAVEL XR	amphetamine	Tris	Attention Deficit Hyperactivity Disorder	Oral	03-2027
TUDORZA PRESSAIR	aclidinium	AstraZeneca	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
DUAKLIR PRESSAIR	aclidinium/formoterol fumarate	AstraZeneca	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027
MOXATAG	amoxicillin	Vernalis	Tonsillitis/Pharyngitis	Oral	05-2027
RAPIVAB	peramivir	BioCryst	Treatment of Acute Uncomplicated Influenza	Intravenous	05-2027
AVEED	testosterone undecanoate	Endo	Testosterone Replacement	Intramuscular	05-2027
NUCYNTA ER	tapentadol	Collegium	Moderate to Severe Chronic Pain	Oral	06-2027
LUMIGAN	bimatoprost	Allergan/AbbVie	Glaucoma; Ocular Hypertension	Ophthalmic	06-2027
ORENITRAM	treprostinil diolamine	Supernus/United Therapeutics	Pulmonary Arterial Hypertension	Oral	06-2027
PLEGRIDY	peginterferon beta-1a	Biogen	Relapsing-Remitting Multiple Sclerosis	Subcutaneous	06-2027
XTANDI	enzalutamide	Astellas/Pfizer	Prostate Cancer	Oral	3Q-2027
RELISTOR	methylnaltrexone	Bausch Health	Opioid-Induced Constipation	Subcutaneous	07-2027
MYALEPT	metreleptin	Aegerion	Leptin Deficiency in Patients with Lipodystrophy	Subcutaneous	07-2027
DOPTELET	avatrombopag	AkaRx	Thrombocytopenia	Oral	07-2027
PIZENSY	lactitol	Braintree/Sebela	Chronic Idiopathic Constipation	Oral	08-2027
CRESEMBA	isavuconazonium	Astellas	Invasive Aspergillosis; Invasive Mucormycosis	Oral	09-2027
SOLOSEC	secnidazole	Lupin	Bacterial Vaginosis; Trichomoniasis	Oral	09-2027
GRASTEK	timothy grass pollen allergen extract	ALK-Abello/Catalent	Grass Pollen-Induced Allergic Rhinitis	Sublingual	4Q-2027
BRONCHITOL	mannitol	Arna Pharma	Cystic Fibrosis	Inhalation	10-2027
TALICIA	amoxicillin/rifabutin/omeprazole	Redhill Biopharma	Helicobacter pylori	Oral	11-2027
FANAPT	iloperidone	Vanda	Schizophrenia; Bipolar Disorder	Oral	11-2027
NUCALA	mepolizumab	GSK	Severe Asthma; Rhinosinusitis with Nasal Polyps; Eosinophilic Granulomatosis with Polyangitis; Hypereosinophilic Syndrome	Subcutaneous	11-2027
ZOKINVY	lonafarnib	Sentynl Therapeutics	Hutchinson-Gilford Progeria Syndrome	Oral	11-2027
TRULICITY	dulaglutide	Eli Lilly	Type 2 Diabetes Mellitus	Subcutaneous	12-2027
INCRUSE ELLIPTA	umeclidinium	GSK	Chronic Obstructive Pulmonary Disease	Oral	12-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
ZONTIVITY	vorapaxar sulfate	Key Pharma	Reduction of Thrombotic Cardiovascular Events in Patients with a History of Myocardial Infarction or with Peripheral Arterial Disease	Oral	12-2027
ADYNOVATE	antihemophilic factor recombinant pegylated	Takeda	Hemophilia A	Intravenous	12-2027
2028 Possible launch date (1st half)					
TRIUMEQ	abacavir/dolutegravir/lamivudine	ViiV Healthcare	Human Immunodeficiency Virus Infection	Oral	2028
KALYDECO	ivacaftor	Vertex	Cystic Fibrosis	Oral	2028
TIVICAY	dolutegravir	ViiV Healthcare	Human Immunodeficiency Virus Infection	Oral	2028
ENTYVIO	vedolizumab	Takeda	Ulcerative Colitis; Crohn's Disease	Intravenous; subcutaneous	2028
KEYTRUDA	pembrolizumab	Merck	Cancer	Intravenous	2028
NEXTSTELLIS	drospirenone/estetrol	Mayne	Prevention of Pregnancy	Oral	2028
EDARBYCLOR	azilsartan kamedoxomil/chlorthalidone	Azurity Pharmaceuticals	Hypertension	Oral	01-2028
INLYTA	axitinib	Pfizer	Advanced Renal Cell Carcinoma	Oral	01-2028
GILOTRIF	afatinib	Boehringer Ingelheim	Non-Small Cell Lung Cancer	Oral	01-2028
ENSTILAR	betamethasone dipropionate/calcipotriol	LEO Pharma	Plaque Psoriasis	External	01-2028
INVOKANA	canagliflozin	Janssen/Vifor	Type 2 Diabetes Mellitus (T2DM); Reduce the Risk of Major Adverse Cardiovascular (CV) Events in Patients with CV Disease; Reduce the Risk of End-Stage Kidney Disease and CV events in Adults with T2DM and Chronic Kidney Disease	Oral	01-2028
GLOPERBA	colchicine	Scilex	Prophylaxis of Gout Flares	Oral	01-2028
ONUREG	azacitidine	Celgene/Bristol-Myers Squibb	Acute Myeloid Leukemia	Oral	01-2028
XIFAXAN	rifaximin	Bausch Health	Traveler's Diarrhea; Hepatic Encephalopathy; Irritable Bowel Syndrome With Diarrhea	Oral	01-2028
WAKIX	pitolisant	Harmony Biosciences	Narcolepsy	Oral	01-2028
INVOKAMET XR	canagliflozin/metformin	Janssen	Type 2 Diabetes Mellitus; Reduce the Risk of Major Adverse Cardiovascular Events in Patients with Cardiovascular Disease	Oral	01-2028

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
MEKINIST	trametinib dimethyl sulfoxide	Novartis/GSK	Melanoma; Non-Small Cell Lung Cancer; Anaplastic Thyroid Cancer; Glioma; Solid Tumors	Oral	01-2028
INVOKAMET	canagliflozin/metformin	Janssen	Type 2 Diabetes Mellitus; Reduce the Risk of Major Adverse Cardiovascular Events in Patients with Cardiovascular Disease	Oral	01-2028
KYBELLA	deoxycholic acid	Allergan	Improvement in the Appearance of Moderate to Severe Convexity or Fullness Associated with Submental Fat in Adults	Subcutaneous	01-2028
THYQUIDITY	levothyroxine	Vistapharm	Hypothyroidism; Pituitary Thyrotropin Suppression	Oral	01-2028
YUTIQ	fluocinolone	Alimera Sciences	Non-Infectious Uveitis	Intravitreal	01-2028
NAYZILAM	midazolam	UCB	Epilepsy	Intranasal	01-2028
NOURIANZ	istradefylline	Kyowa Kirin	Parkinson's Disease	Oral	01-2028
ZETONNA	ciclesonide	Covis Pharma	Seasonal and Perennial Allergic Rhinitis	Intranasal	02-2028
PERSERIS	risperidone	Indivior	Schizophrenia	Subcutaneous	02-2028
WINLEVI	clascoterone	Cassiopea/Cosmo Technologies	Acne Vulgaris	External	02-2028
KOVALTRY	antihemophilic factor recombinant	Bayer	Hemophilia A	Intravenous	03-2028
ZOLINZA	vorinostat	Merck	Cutaneous T-Cell Lymphoma	Oral	03-2028
CINQAIR	reslizumab	Teva Respiratory	Severe Asthma	Intravenous	03-2028
ELIQUIS	apixaban	Pfizer/Bristol-Myers Squibb	Reduce the Risk of Stroke and Systemic Embolism in Patients with Nonvalvular Atrial Fibrillation; Prophylaxis of Deep Vein Thrombosis (DVT) in Patients Who Have Undergone Hip or Knee Replacement Surgery; Treatment of DVT and PE	Oral	04-2028
DALVANCE	dalbavancin	AbbVie	Acute Bacterial Skin and Skin Structure Infections	Intravenous	05-2028
VERKAZIA	cyclosporine	Santen	Vernal Keratoconjunctivitis	Ophthalmic	06-2028
VYONDYS 53	golodirsen	Sarepta Therapeutics	Duchenne Muscular Dystrophy	Intravenous	06-2028

References:

AbbVie Press Release. AbbVie Web site. AbbVie submits Biologics License Application to the FDA for American Lung Association. American Lung Association Web site. Bronchiectasis. https://www.lung.org/lung-health-diseases/lung-disease-lookup/bronchiectasis. Accessed March 27, 2025.

Bekker LG, Das M, Karim QA, et al; PURPOSE 1 Study Team. Twice-Yearly Lenacapavir or Daily F/TAF for HIV Prevention in Cisgender Women. N Engl J Med. 2024 Oct 3;391(13):1179-1192.

BioMedTracker Drug Intelligence Platform. BioMedTracker Web site. http://www.biomedtracker.com.

Centers for Disease Control and Prevention (CDC). CDC Web site. Clinical guidance for PrEP. https://www.cdc.gov/hivnexus/hcp/prep/index.html. February 10, 2025. Accessed April 8, 2025.

Capricor Investor Presentation. Capricor Web site. Capricor corporate presentation. https://d1io3yog0oux5.cloudfront.net/_6c84c9259ee6d3b6cb65cfcd87db85aa/capricor/db/2227/21450/pdf/CAPR+March+2025.pdf. March 11, 2025. Accessed March 23, 2025.

Capricor Therapeutics Press Release. Capricor Web site. Capricor Therapeutics announces FDA acceptance and Priority Review of its Biologics License Application for deramiocel to treat Duchenne muscular dystrophy. https://www.capricor.com/investors/news-events/press-releases/detail/305/capricor-therapeutics-announces-fda-acceptance-and-priority. March 4, 2025. Accessed March 23, 2025.

Capricor Therapeutics Press Release. Capricor Web site. Capricor Therapeutics announces long-term benefit of deramiocel (CAP-1002) in both skeletal muscle and cardiac function in the HOPE-2 OLE study in Duchenne muscular dystrophy. https://www.capricor.com/investors/news-events/press-releases/detail/284/capricor-therapeutics-announces-long-term-benefit-of. June 28, 2024. Accessed April 7, 2025.

Cohn DM, Bordone L, Newman KB, et al. Donidalorsen for the treatment of hereditary angioedema: Results from a phase 3, randomised, placebo-controlled trial. Poster presented at: European Academy of Allergy and Clinical Immunology (EAACI) Congress; May 31-June 3, 2024; Valencia, Spain.

Crinetics Pharmaceuticals. Crinetics Web site. Let's talk about acromegaly. https://crinetics.com/acromegaly-symptoms-diagnosis-treatment-resources-2/#epidemiology. 2025. Accessed April 16, 2025.

Crinetics Pharmaceuticals Corporate Presentation. Crinetics Web site. Corporate presentation. https://ir.crinetics.com/static-files/f168e155-6462-4d0f-92c8-64603d610397. March 2025. Accessed April 8, 2025.

Crinetics Pharmaceuticals Corporate Presentation. Crinetics Web site. Crinetics topline results from paltusotine phase 3 PATHFNDR-1 study. https://crinetics.com/paltusotine-phase3-pathfndr1-study-topline-results/. September 11, 2023. Accessed April 8, 2025.

Crinetics Pharmaceuticals Corporate Presentation. Crinetics Web site. Topline results from paltusotine phase 3 PATHFNDR-2 study. https://crinetics.com/topline-results-from-paltusotine-phase-3-pathfndr-2-study/. March 19, 2024. Accessed April 8, 2025.

Crinetics Pharmaceuticals Press Release. Crinetics Web site. Crinetics announces FDA acceptance of New Drug Application for paltusotine for adult patients with acromegaly. https://crinetics.com/crinetics-announces-fda-acceptance-of-new-drug-application-for-paltusotine-for-adult-patients-with-acromegaly/. December 9, 2024. Accessed April 8, 2025.

Fox RJ, Bar-Or A, Traboulsee A, et al; HERCULES Trial Group. Tolebrutinib in nonrelapsing secondary progressive multiple sclerosis. N Engl J Med. 2025. Epub ahead of print.

Gilead Press Release. Gilead Web site. U.S. FDA accepts Gilead's New Drug Applications for twice-yearly lenacapavir for HIV prevention under Priority Review. https://www.gilead.com/news/news-details/2025/us-fda-accepts-gileads-new-drug-applications-for-twice-yearly-lenacapavir-for-hiv-prevention-under-priority-review. February 18, 2025. Accessed April 8, 2025.

Gilead Press Release. Gilead Web site. First clinical data for Gilead's investigational once-yearly lenacapavir for HIV prevention presented at CROI 2025 and published in The Lancet. https://www.gilead.com/news/news-details/2025/first-clinical-data-for-gileads-investigational-once-yearly-lenacapavir-for-hiv-prevention-presented-at-croi-2025-and-published-in-the-lancet. March 11, 2025. Accessed April 13, 2025.

HIV.gov. HIV.gov Web site. U.S. statistics. https://www.hiv.gov/hiv-basics/overview/data-and-trends/statistics. February 21, 2025. Accessed April 8, 2025.

Insmed Press Release. Insmed Web site. Additional positive data from pivotal ASPEN study of brensocatib in patients with bronchiectasis to be presented at the 7th World Bronchiectasis Conference. https://investor.insmed.com/2024-07-03-Additional-Positive-Data-from-Pivotal-ASPEN-Study-of-Brensocatib-in-Patients-with-Bronchiectasis-to-be-Presented-at-the-7th-World-Bronchiectasis-Conference. July 3, 2024. Accessed March 27, 2025.

Insmed Press Release. Insmed Web site. FDA grants Priority Review to Insmed's brensocatib for treatment of bronchiectasis with PDUFA target action date set for August 12, 2025. https://investor.insmed.com/2025-02-06-FDA-Grants-Priority-Review-to-Insmeds-Brensocatib-for-Treatment-of-Bronchiectasis-with-PDUFA-Target-Action-Date-Set-for-August-12,-2025. February 6, 2025. Accessed March 26, 2025.

Ionis Pharmaceuticals Corporate Presentation. Ionis Web site. Company presentation. https://ir.ionis.com/static-files/21bb1591-8b81-4eab-bf68-19a5e39e7e5b. March 2025. Accessed April 10, 2025.

Ionis Pharmaceuticals Press Release. Ionis Web site. Ionis announces FDA acceptance of New Drug Application for donidalorsen for prophylactic treatment of HAE. https://ir.ionis.com/news-releases/news-release-details/ionis-announces-fda-acceptance-new-drug-application-donidalorsen. November 4, 2024. Accessed April 10, 2025.

Kelley CF, Acevedo-Quinones M, Agwu AL, et al; PURPOSE 2 Study Team. Twice-yearly lenacapavir for HIV prevention in men and gender-diverse persons. N Engl J Med. 2025 Apr 3;392(13):1261-1276.

Kosiborod MN, Abildstrøm SZ, Borlaug BA, et al; STEP-HFpEF Trial Committees and Investigators. Semaglutide in patients with heart failure with preserved ejection fraction and obesity. N Engl J Med. 2023;389(12):1069-1084.

Kosiborod MN, Petrie MC, Borlaug BA, et al; STEP-HFpEF DM Trial Committees and Investigators. Semaglutide in patients with obesity-related heart failure and type 2 diabetes. N Engl J Med. 2024;390(15):1394-1407.

Kosiborod MN, Deanfield J, Pratley R, et al; SELECT, FLOW, STEP-HFpEF, and STEP-HFpEF DM Trial Committees and Investigators. Semaglutide versus placebo in patients with heart failure and mildly reduced or preserved ejection fraction: a pooled analysis of the SELECT, FLOW, STEP-HFpEF, and STEP-HFpEF DM randomised trials. Lancet. 2024;404(10456):949-961.

Long-term outcomes of ataluren in Duchenne muscular dystrophy. ClinicalTrials.gov identifier: NCT03179631. Updated January 18, 2024. Accessed April 13, 2025. https://www.clinicaltrials.gov/study/NCT03179631.

McDonald CM, Wu S, Gulati S, et al. Safety and efficacy of ataluren in nmDMD patients from Study 041, a phase 3, randomized, double-blind, placebo-controlled trial (PL5.001). Neurology. 2023 Apr 25;100(17):S2.

McDonald CM, Marban E, Hendrix S, et al; HOPE-2 Study Group. Repeated intravenous cardiosphere-derived cell therapy in late-stage Duchenne muscular dystrophy (HOPE-2): a multicentre, randomised, double-blind, placebo-controlled, phase 2 trial. Lancet. 2022 Mar 12;399(10329):1049-1058.

Mercuri E, Osorio AN, Muntoni F, et al; STRIDE and CINRG DNHS investigators. Safety and effectiveness of ataluren in patients with nonsense mutation DMD in the STRIDE Registry compared with the CINRG Duchenne Natural History Study (2015–2022): 2022 interim analysis. J Neurol. 2023 Apr 28;270:3896-3913.

National Institute of Neurological Disorders and Stroke. Multiple sclerosis. https://www.ninds.nih.gov/health-information/disorders/multiple-sclerosis. Accessed April 3, 2025.

National Organization for Rare Disorders (NORD). NORD Web site. Acromegaly. https://rarediseases.org/rare-diseases/acromegaly/. February 24, 2025. Accessed April 8, 2025.

National Organization for Rare Disorders (NORD). NORD Web site. Duchenne muscular dystrophy. https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy. July 15, 2024. Accessed April 5, 2025.

National Organization for Rare Disorders (NORD). NORD Web site. Hereditary angioedema. https://rarediseases.org/rarediseases/hereditary-angioedema. January 6, 2025. Accessed March 28, 2025.

PTC Therapeutics Presentation. PTC Web site. Translarna™ (Ataluren) Study 041 topline results. https://ir.ptcbio.com/events-presentations. June 21, 2022. Accessed April 13, 2025.

PTC Therapeutics Presentation. PTC Web site. 2025 J.P. Morgan Healthcare Conference presentation. https://ir.ptcbio.com/events-presentations. January 29, 2025. Accessed April 9, 2025.

PTC Therapeutics Press Release. PTC Web site. PTC announces results from Phase 3 ACT DMD clinical trial of Translarna™ (ataluren) in patients with Duchenne muscular dystrophy. https://ir.ptcbio.com/news-releases/news-release-details/ptc-announces-results-phase-3-act-dmd-clinical-trial. October 15, 2015. Accessed April 9, 2025.

PTC Therapeutics Press Release. PTC Web site. PTC Therapeutics announces FDA acceptance of Translarna™ NDA resubmission. https://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-announces-fda-acceptance-translarnatm-nda. October 30, 2024. Accessed April 9, 2025.

PTC Therapeutics Press Release. PTC Web site. PTC Therapeutics provides regulatory update on Translarna™ (ataluren) in Europe. https://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-provides-regulatory-update-translarnatm-1. March 28, 2025. Accessed April 9, 2025.

Riedl MA, Bordone L, Tachdjian R, et al. Self-reported treatment preferences of patients switching from prior prophylactic therapies to donidalorsen for the treatment of hereditary angioedema. Poster presented at: EAACI Congress; May 31-June 3, 2024; Valencia, Spain.

Riedl MA, Tachdjian R, Lumry WR, et al; OASIS-HAE Team. Efficacy and safety of donidalorsen for hereditary angioedema. N Engl J Med. 2024 May 31;391:21-31.

Sanofi Press Release. Sanofi Web site. Press Release: Tolebrutinib demonstrated a 31% delay in time to onset of confirmed disability progression in non-relapsing secondary progressive multiple sclerosis phase 3 study. https://www.sanofi.com/en/media-room/press-releases/2024/2024-09-20-09-30-00-2949552. September 20, 2024. Accessed April 3, 2025.

Sanofi Press Release. Sanofi Web site. Press Release: Tolebrutinib regulatory submission accepted for priority review in the US for patients with multiple sclerosis. https://www.sanofi.com/en/media-room/press-releases/2025/2025-03-25-06-00-00-3048411. March 25, 2025. Accessed April 3, 2025.

Tachdjian R, Riedl MA, Bordone L, et al. Long-term safety of donidalorsen for the treatment of hereditary angioedema. Poster presented at: EAACI Congress; May 31-June 3, 2024; Valencia, Spain.

UpToDate Database. https://www.uptodate.com.

U.S. Preventive Services Task Force. Preexposure prophylaxis to prevent acquisition of HIV: U.S. Preventive Services Task Force Recommendation Statement. JAMA. 2023 Aug 22;330(8):736-745.

Optum Rx®

optum.com/optumrx