Maxor®

PHARMACY BENEFIT MANAGEMENT SERVICES

MAXOR PIPELINE Q2 2025

Pipeline news | New to market brands and generics | Drug pipeline spotlight

April 2025

SPOTLIGHT | PIPELINE NEWS

FDA clarifies policies for GLP-1 compounding

- On March 10, 2025, the Food and Drug Administration (FDA) determined that the shortage of semaglutide injection products, which are glucagon-like peptide (GLP-1) medications, is resolved. Semaglutide is marketed under the brand names Wegovy® and Ozempic®.
- While intermittent supply disruptions may still occur as the products move through the supply chain, the drug's manufacturer has confirmed that availability can meet the present and projected national demand.
- With shortages resolving, compounded formulations of semaglutide will no longer be permitted. The FDA issued guidance that requires compounding facilities to cease production by the end of April 2025 or May 2025 depending on the type of license held by the facility.

Epinephrine nasal spray receives approval for pediatric patients

- On March 5, 2025, Neffy® 1mg (epinephrine nasal spray) was approved for the treatment of type 1 allergic reactions, including anaphylaxis, in pediatric patients 4 years of age and older weighing 33 pounds to <66 pounds.
- This marks the first approval of a new epinephrine delivery method for this population in over 35 years. Neffy 2mg is approved for patients weighing 66 pounds or more.
- Neffy 1mg is expected to be priced identically to the 2mg dose ($710 WAC for a two-pack). This price is higher compared to injectable epinephrine products but comes with a longer shelf life and easy-to-understand administration.
- Neffy 1mg is expected to be launched in the United States in May 2025.

2024 novel drug approval highlights

- A novel drug is defined by the FDA as a drug with an active ingredient that has never been approved or marketed before in the United States.
- About 30% of the approvals from 2024 have sales predictions that approach $1 billion or higher. These include Amtagvi® for melanoma, Cobenfy® for schizophrenia, and Itovebi® for breast cancer.
- Gene therapies made up 7% of approvals and included the two highest-cost drugs ever launched, Lenmeldy® and Kebilidi®. Additional expenses such as pre-medications, administration fees, and hospital stays that are part of gene-therapy administration are not included in the drug cost and can vary widely depending on the treatment.

Newly approved gene therapies

Drug name	Approval date	Indication	Product insights
Lenmeldy [Orchard]	3/18/2024	MLD	First gene therapy for this indication; $4.2M therapy cost
Kebilidi [PTC Therapeutics]	11/13/2024	AADC deficiency	First gene therapy for this indication; $3.9M therapy cost
Tecelra [Xeno Bioscience]	8/1/2024	Synovial sarcoma	First FDA-approved T-cell receptor gene therapy; $763,000 therapy cost
Beqvez [Pfizer]	4/25/2024	Hemophilia B	In February 2025, Pfizer terminated development citing limited patient and provider interest

Source: IPD Analytics
Abbreviations: MLD, metachromatic leukodystrophy; AADC, aromatic L-amino acid decarboxylase

NOW APPROVED | NEW BRAND DRUGS TO MARKET

Brand name	Generic name	Indication	ROA	Approval month
ALYFTREK™	Vanzacaftor-Tezacaftor-Deutivacaftor	Cystic fibrosis	OR	Jan-25
KEBILIDI™	Eladocagene Exuparvovec	Aromatic l-amino acid decarboxylase deficiency	IJ	Jan-25
OPDIVO QVANTIG™	Nivolumab-Hyaluronidase	Cancer	SC	Jan-25
ALHEMO®	Concizumab	Hemophilia A; Hemophilia B	SC	Feb-25
DATROWAY®	Datopotamab Deruxtecan	Breast cancer	IV	Feb-25
PREVYMIS®	Letermovir	Prevention of cytomegalovirus diseases	OR	Feb-25
STEQEYMA®	Ustekinumab	Chronic inflammatory diseases	IV	Feb-25
YESINTEK™	Ustekinumab	Chronic inflammatory diseases	SC	Feb-25
JOURNAVX™	Suzetrigine	Acute pain	OR	Feb-25
NIKTIMVO™	Axatilimab	Graft versus host disease	IV	Feb-25
EVRYSDI®	Risdiplam	Spinal muscular atrophy	OR	Mar-25
GOMEKLI™	Mirdametinib	Neurofibromatosis type 1	OR	Mar-25
GRAFAPEX™	Treosulfan	Allogeneic hematopoietic stem cell transplantation	IV	Mar-25
ONAPGO™	Apomorphine	Parkinson's disease	SC	Mar-25
OTULFI®	Ustekinumab	Chronic inflammatory diseases	SC	Mar-25
PYZCHIVA®	Ustekinumab	Chronic inflammatory diseases	SC	Mar-25
SELARSDI™	Ustekinumab	Chronic inflammatory diseases	SC	Mar-25
BKEMV™	Eculizumab	Paroxysmal nocturnal hemoglobinuria	IV	Mar-25
ROMVIMZA™	Vimseltinib	Tenosynovial giant cell tumor	OR	Mar-25
VIMKUNYA™	Chikungunya vaccine	Chikungunya	IM	Mar-25

NOW APPROVED | NEW GENERIC DRUGS TO MARKET

Brand name	Generic name	Indication	Launch month
SANDOSTATIN LAR®	Octreotide	Acromegalic, carcinoid rumors, vasoactive intestinal peptide tumors	Oct-24
TAZORAC®	Tazarotene	Plaque psoriasis	Oct-24
STENDRA®	Avanafil	Erectile dysfunction	Nov-24
BYETTA®	Exenatide	Type 2 diabetes	Nov-24
BETIMOL®	Timolol	Elevated intraocular pressure	Dec-24
MOTEGRITY®	Prucalopride	Chronic idiopathic constipation	Jan-25
NEXIUM®	Esomeprazole	Gastroesophageal reflux disease	Jan-25
ENTRESTO®	Sacubitril-Valsartan	Heart failure	Feb-25
SPRITAM®	Levetiracetam	Seizures	Feb-25
MESNEX®	Mesna	Reducing the incidence of ifosfamide-induced hemorrhagic cystitis	Feb-25
RIDAURA®	Auranofin	Active classical or definite rheumatoid arthritis	Mar-25
IMPOYZ®	Clobetasol	Moderate to severe plaque psoriasis	Mar-25
PURIXAN®	Mercaptopurine	Acute lymphoblastic leukemia	Mar-25
XARELTO®	Rivaroxaban	Thromboembolic disorders	Mar-25

The report provided is for informational purposes only. This information should not be solely relied upon for formulary decision-making purposes and is subject to change. www.maxorplus.com
Abbreviation: ROA- Route of Administration; EX-External; IJ-Injection; IM-Intramuscular; IV-Intravenous; OR-Oral; SC-Subcutaneous; IZ-Intravitreal; IO- Intraocular; IN-Intranasal

CLINICAL PIPELINE

Hereditary angioedema (HAE)

Sebetralstat [KalVista Pharmaceuticals]

- Oral plasma kallikrein inhibitor for on-demand treatment of HAE attacks in patients aged 12 years and older
- Granted FDA Fast Track designation
- If approved, sebetralstat would offer an oral option in comparison to current available therapies that are administered intravenously (Ruconest® and Berinert®) or subcutaneously (Firazyr® and Kalbitor®)
- Estimated WAC expected to be close to $240,000 a year. Pricing would be affected if two doses are needed to treat an attack.
- Regulatory decision expected by June 17, 2025

Myasthenia gravis

Vyvgart Hytrulo PFS [Argenx]

- Currently available as a subcutaneous infusion that is administered by a healthcare professional for the treatment of generalized myasthenia gravis (gMG) and chronic inflammatory demyelinating polyneuropathy (CIDP)
- First self-administered treatment for CIDP and gMG
- Annual price expected to be $300,000 to $500,000 and gives the option to move to the pharmacy benefit from medical with the possibility of bringing plan savings through the alternate site of care
- Approved on April 10, 2025

Pyruvate dehydrogenase complex deficiency (PDCD)

Sodium dichloroacetate [Saol Therapeutics]

- PDCD is a rare disorder (1:50,000) of carbohydrate metabolism caused by a deficiency of one of three enzymes
- Submitted to FDA for use with a proprietary genetic test intended to provided individualized dosing and reduce adverse events
- If approved, would be the first FDA-approved treatment for PDCD
- Granted Orphan Drug, Fast Track, and Rare Pediatric Disease designations by the FDA
- Annual treatment cost estimated to be between $750,000 to $1,000,000
- Regulatory decision expected by May 27, 2025

Pre-exposure prophylaxis (PrEP) for HIV

Lencapavir [Gilead]

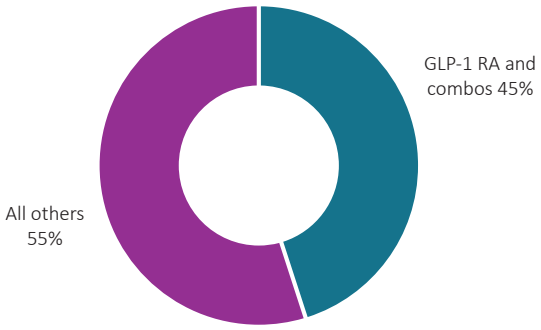
- First-in-class inhibitor of HIV-1 capsid function currently approved for treatment of resistant HIV
- If approved, the twice-yearly injection schedule for lencapavir would compete with Viiv's Apretude, which requires injections every other month, as well as oral options Descovy® and generic Truvada®
- Annual price estimated to be $20,000 to $50,000 for the injection, compared to ~$200 for generic Truvada
- Regulatory decision expected by June 19, 2025

SPOTLIGHT **WEIGHT MANAGEMENT PIPELINE**

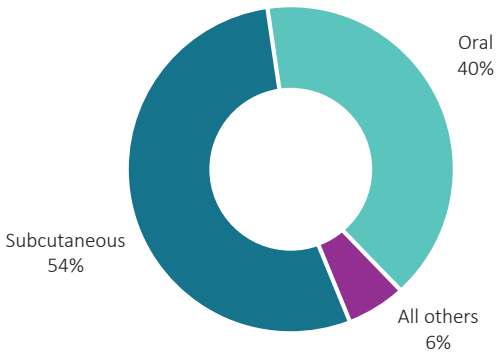
Weight management pipeline continues robust growth

- Glucagon-like peptide 1 receptor agonists (GLP-1 RA) have become increasingly popular for their effectiveness in managing obesity. The drug development pipeline includes over **160 products** to treat obesity that span all phases of the development and approval process.
- In addition to GLP-1 RA, **new mechanisms of action** are being studied that target different receptors in the body, some focusing on weight loss with others focusing on weight maintenance
- **Route of administration** is also a differentiator in the pipeline. Over half of the products are delivered via subcutaneous injection with oral, intravenous, and other methods making up the rest.

Mechanisms of action



Administration



Developments in weight management

New mechanisms are being studied that manage the diagnosis of obesity from many different angles:

- Myostatin inhibitor – maintain muscle mass during weight loss
- TGF-beta inhibitor – monoclonal antibody that binds to specific receptors
- Oxygen diffusion enhancing compound – engineered to mimic the gut microbiome changes induced by gastric bypass surgery
- Cannabinoid receptor antagonist – affects metabolic regulation

Weight management drugs in pipeline

Drug name	Mechanism of action	Route of administration	Development phase
Apitegromab [Scholar Rock]	Myostatin inhibitor	Intravenous	Phase II
Bimagrumab [Eli Lilly]	TGF-beta inhibitor	Intravenous	Phase II
Sodium percarbonate [Xeno Biosciences]	Oxygen diffusion-enhancing compound	Oral	Phase I
Monlunabant [Novo Nordisk]	Cannabinoid receptor antagonist	Oral	Developing

Source: IPD Analytics

SPOTLIGHT
OPHTHALMIC CONDITIONS

Options for rare ophthalmic conditions expected to expand

- Many rare ophthalmic conditions that can lead to vision impairment or blindness have gone years with a lack of treatment options.
- Drug development has put focus on this area which has led to many potential approvals in the next one to two years.
- These therapies range from one-time gene therapies to orally administered products that are used for chronic treatment.

Encelto is first FDA-approved treatment for MacTel type 2

- On March 5, 2025, Encelto™ became the first FDA-approved therapy for macular telangiectasia (MacTel) type 2, a rare eye disease that leads to progressive and irreversible vision loss.
- In the United States, MacTel type 2 has a prevalence of about 0.1% in people over age 40 and is more common than type 1.
- Encelto is a cell-based therapy that is delivered via surgical intravitreal implantation and is recommended to be implanted into each affected eye.

Cost estimated at
$200,000 to $500,000
per treated eye

Therapies in late-stage development

- If approved, these treatments are expected to be very costly, ranging from $100,000 annually to $1,000,000 for one-time therapy. Many may require complex distribution and administration processes.

Rare ophthalmic conditions in pipeline

Drug name	Indication	Pipeline insights	Pending approval date
Gildeuretinol [Alkeus Pharma]	Stargardt disease	Oral therapy; estimated annual price $100,000-$300,000	Late 2025/early 2026
Gene therapy **Botaretigene sparoparvovec [MeiraGTx/J&J]**	XLRP	Subretinal; estimated $750,000-$1,000,000 one time therapy	Late 2025/early 2026
Gene therapy **Sonpiretigene isteparvovec [Nanoscope]**	RP (all forms)	Intravitreal; estimated $750,000-$1,000,000 one time therapy	Late 2025/early 2026
Tinlarebant [Belite Bio]	Stargardt disease	Oral therapy; estimated annual price $300,000-$500,000	2026

*IPD Analytics, December 2024
Abbreviations:Mac-Tel, macular telangiectasia; XLRP, X-linked retinitis pigmentosa; RP, retinitis pigmentosa;