WINTER FDA APPROVALS

Select Highlights

Anchor Point Insights

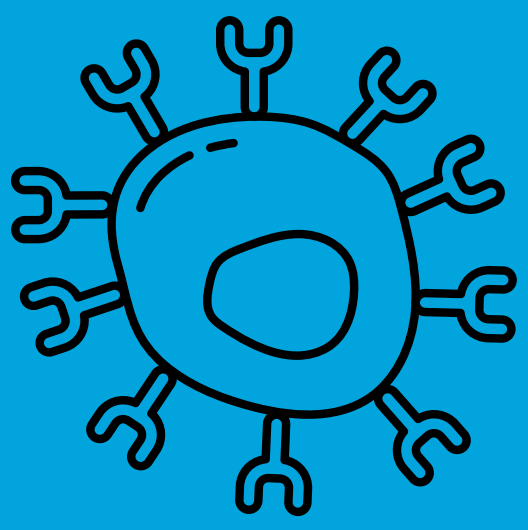
Autolus

Nov 08, 2024

AUCATZYL

(obecabtagene autoleucel)

For the treatment of adults with **relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)**



410x10^6 CD19 CAR+ T-cells as split-dose infusion on Day 1 and Day 10 (±2 days)

- Aucatzyl is an autologous CD19-directed CAR-T cell therapy with approval based on the Ph1/2 FELIX study which demonstrated a **63% OCR, with 42% acheiving CR within 3 mos, and a mDOR of 14.1 mos**
- Safety outcomes include a boxed warning for **CRS (3% Gr3)**, neurologic toxicities (**7% Gr≥3 ICANS**), and secondary hematologic malignancies
- Aucatzyl has been priced at **$525,000, which is comparable to other CAR-T treatments** for R/R B-ALL including Novartis' Kymriah ($580,0000) and Gilead's Tecartus ($460,000)

<u>API Take:</u> As the first CAR-T therapy approved without a Risk Evaluation and Mitigation Strategy (REMS) program, Autolus will potentially rely on safety messaging to serve as a differentiator over established therapies.

Anchor Point Insights

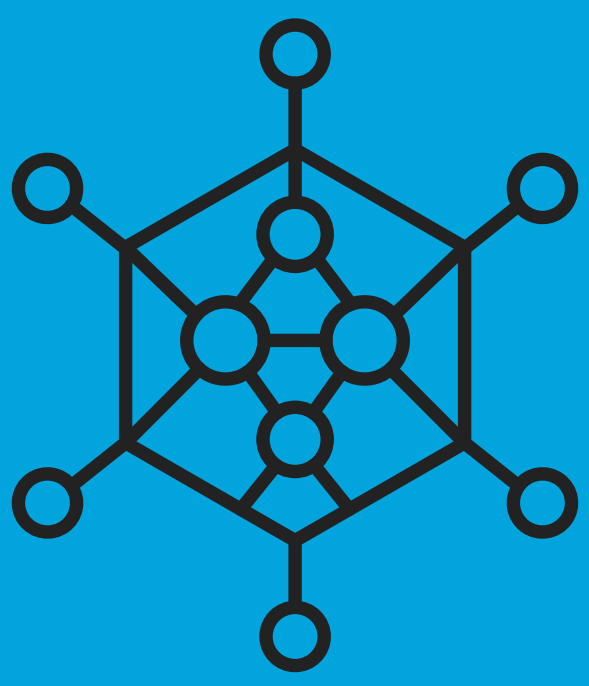
PTC THERAPEUTICS™

Nov 13, 2024

KEBILIDI

(eladocagene exuparvovec-tneq)

For the treatment of adult and pediatric patients with **aromatic L-amino acid decarboxylase (AADC) deficiency**



One-time delivery directly to brain via non-invasive surgical procedure

- recombinant adeno-associated virus serotype 2 (rAAV2)-based gene therapy, containing the human dopa decarboxylase (DDC) gene that **restores enzymatic function, dopamine metabolism, and motor function in an otherwise fatal disease**
- Following Priority Review, approval is based on results from a Ph1/2 trial that demonstrated **clinical benefit as early as 3 months after administration, and sustained for at least 10 years**
- The asset has already been approved in the EU, U.K, and select other countries for considerable time under the brand name **Upstaza**, **where it costs ~$3.75M**

<u>API Take:</u> As the first FDA approved gene therapy to be delivered directly to the brain, Kebilidi's approval is encouraging to the 500+ gene therapies in development. With a well-tolerated safety profile, remarkable clinical benefit, and approval for patients of any disease severity, Kebilidi may be a life-changing therapy for AADC patients.

Anchor Point Insights

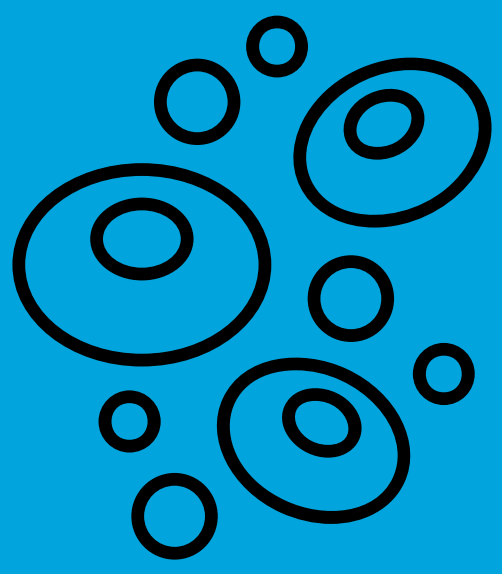
mesoblast

Dec 18, 2024

RYONCIL

(remestemcel-L)

For the treatment of **steroid-refractory acute graft versus host disease (SR-aGVHD)** in pediatric patients 2 months of age and older



2x10^6 MSC/kg body weight 2x/week for 4 weeks (8 total infusions)

- Ryoncil is the **first FDA approved allogeneic bone marrow-derived mesenchymal stromal cell (MSC) therapy**
- Approval is based on data from a Ph3 single-arm study (MSB-GVHD001) that demonstrated a **70% ORR (30% CR) at Day 28 and a mDOR of 54 days**
- Ryoncil had previously been **rejected twice** by the FDA: first, in 2020 due to a request for an additional trial; and then a resubmission in 2023 for a request for addtional data in adult patients

<u>API Take:</u> Following an arduous journey, approval of the first MSC therapy is notable in its own right. As the only approved therapy for SR-aGVHD, Ryoncil offers promising efficacy and safety for pediatric patients with few treatment alternatives.

Anchor Point Insights

Lilly

FDA APPROVED

Dec 20, 2024

ZEPBOUND

(tirzepatide)

For the treatment of **moderate to severe obstructive sleep apnea (OSA) in adults with obesity,** along with a reduced-calorie diet and increased physical activity

Injected once a week, subcutaneously

- Zepbound has now become **the first drug treatment option for certain patients with OSA**
- Tirzepatide **reduced symptoms of OPS by nearly two-thirds** in adults with OSA and obesity, and met all primary and key secondary endpoints in two Phase 3 clinical trials
- The Centers for Medicare & Medicaid Services confirmed that **Medicare drug plans would Zepbound for OSA** (Medicare **does not** currently cover Zepbound for weight loss specifically)

<u>API Take:</u> While GLP-1 inhibitors have gained acclaim for their effectiveness in weight loss, their therapeutic potential in addressing secondary conditions linked to obesity has also proven to be transformative. Zepbound offers a much-needed therapeutic option for patients with OSA, a condition with limited treatment alternatives.

Anchor Point Insights

VERTEX®

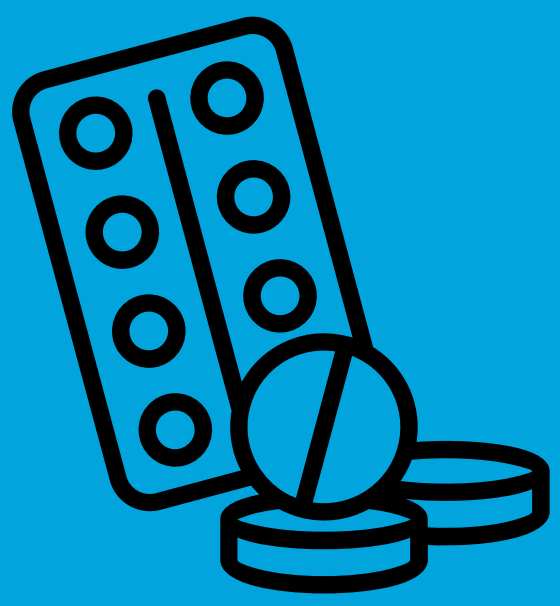
FDA APPROVED

Jan 30, 2025

JOURNAVX

(suzetrigine)

For the treatment of adults with **moderate-to-severe acute pain**



100 mg starting dose, followed by 50 mg every 12 hours

Not studied beyond 14 day use

- JOURNAVX is the **first and only approved non-opioid oral pain signal inhibitor** and the **first new class of pain medicine approved in >20 years**
- Oral, **highly selective pain signal inhibitor** that is selective for NaV1.8, a sodium channel **selectively expressed in peripheral pain-sensing neurons**
- In **two separate trials**, Journavx was found to be **equally as effective as opioids at blocking acute pain** following moderately painful surgery, **with fewer side effects and no risk of addiction**
- **Vertex has established a wholesale acquisition cost of $15.50 per 50mg pill for Journavx in the US**

<u>API Take:</u> While this is a promising step forward in reducing prescription opioid use, there is currently no evidence supporting the use of Journavx for chronic pain relief. With an estimated 20% of Americans experiencing chronic pain, developing non-addictive therapies is a critical area of need.

Anchor Point Insights