SUMMER FDA APPROVALS

Select Highlights

Anchor Point Insights

UTILITY therapeutics

April 24, 2024

PIVYA

(pivmecillinam)

For the treatment of adult females with **uncomplicated urinary tract infections** (UTIs) caused by susceptible isolates of *Escherichia coli, Proteus mirabilis* or *Staphylococcus saprophyticus*

Three times daily oral tablet for 3-7 days

- Pivya is a prodrug of mecillinam, a beta-lactam antibacterial, mainly active against gram negative bacteria
- Approval based on data from 3 trials showing Pivya was **more effective than placebo + ibuprofen, & as effective as cephalexin** in treating UTI symptoms & clearing urine of pathogens
- An IV formulation is being developed as a first-line therapy for complicated UTI in the hospital setting

API Take: Pivya, approved in the EU for >40 years & often recommended as a first-line option, has extensive real world data supporting its safety & efficacy. This **expands treatment options for UTIs in the US, the top cause of ex-hospital antibiotic use.**

Anchor Point Insights

AMGEN

June 12, 2024

IMDELLTRA

(tarlatamab-dlle)

For the treatment of adult patients with **extensive-stage small cell lung cancer (ES-SCLC)** with disease progression on or after platinum-based chemotherapy

1-hour biweekly IV infusion after initial step-up dosing

- **Bispecific antibody (bsAb)** engaging DLL3 on SCLC cells & CD3 on T cells to **kill the tumor** cells
- Acellerated approval based on data from Ph2 DeLLphi-301 study demonstrating a **40% ORR**, mDOR of 9.7mos, and **mOS of 14.3 mos**
- **First approval of a bsAb in a solid tumor setting** as well as the first significant advancement in ES-SCLC treatment following checkpoint inhibitor approvals (which were the first novel approvals in 15+ years)

API Take: While T-cell-engaging therapies (bsAbs, CAR-T, etc.) have made an impact in heme malignancies, this approval represents an **important first in a solid tumor setting.**

Anchor Point Insights

June 20, 2024

PIASKY

(crovalimab-akkz)

For the treatment of pediatric & adult patients (≥ 13 yrs; ≥ 40 kg) with **paroxysmal nocturnal hemoglobinuria (PNH)**

Monthly SC injection following an initial IV infusion and weekly SC loading dose

- **Monoclonal antibody** targeting C5 complement component to reduce hemolysis
- Approval based on data from Ph3 COMMODORE 2 study showing **a 79.3% rate of hemolysis control from week 5 to week 25** demonstrating non-inferiority compared to eculizumab
- **Third FDA approval of a complement C5 inhibitor** following AstraZeneca's (via Alexion aquisition) Soliris (eculizumab) and Ultomiris (ravulizumab), but also competing with recent approvals for alternative complement inhibitors (Sobi's C3 inhibitor Empaveli and Novartis' Factor B inhibitor Fabhalta)

API Take: Using recycling antibody technology, crovalimab can be **given less frequently & potentially self-administered**, but reduced treatment burden may not be a significant enough advantage in an increasingly crowded market.

Anchor Point Insights

July 2, 2024

KISUNLA

(donanemab-azbt)

For the treatment of early symptomatic **Alzheimer's disease**, including **cognitive impairment and dementia**, with confirmed amyloid plaques

once monthly IV infusion, discontinue upon sufficient clearance

- **Monoclonal antibody** targeting amyloid beta that **induces microglial clearance** of plaques in the brain
- Approval based on data from Ph3 TRAILBLAZER-ALZ 2 study showing **35% slowing of cognitive decline & 39% lower risk of progression** compared to placebo
- **Third FDA approval of an amyloid-targeting agent** with Aduhelm & Leqembi from Biogen…*but* Biogen discontinued Aduhelm in January 2024 & Leqembi has been slow to gain traction among prescribers

API Take: While donanemab shows **meaningful efficacy**, **a worrisome safety profile** - *1/3 of patients experience cerebral microbleeds* - sparked hesitations among KOLs. **Extensive imaging recommendations** & limited use of Biogen's similar molecule further dampen excitement.

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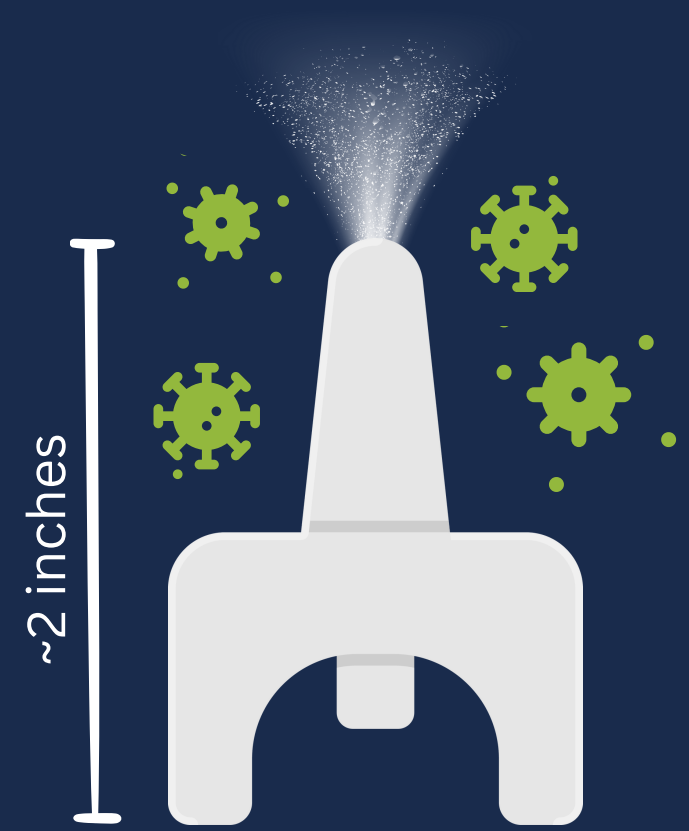
ARS PHARMA

August 9, 2024

NEFFY

(epinephrine nasal spray)

For the treatment of **Type I Allergic Reactions, including anaphylaxis,** in adults & children (≥30 kg)



single dose intranasal spray; 2nd dose, as needed

- Neffy's approval was based on 4 studies in 175 healthy adults without anaphylaxis that demonstrated comparable epinephrine blood concentrations, blood pressure, & heart rate effects to approved epinephrine injection products, with similar results in children
- **The first & only needle-free treatment option for patients living with severe allergic reactions**
- ARS Pharma plans to file a sNDA with the FDA for **children who weigh 15 to <30 kg** by the end of Q3 2024, with **approval by Summer 2025**

API Take: Neffy offers a **potentially game-changing alternative** to the Epi-Pen, with easier delivery, compact size, & low cost. But, with limited data in patients experiencing anaphylaxis, real world outcomes will be critical to validate its efficacy.

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