Evaluate Vantage

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Go or go go? J&J’s cell therapy heads to the FDA

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November approval decisions loom for cilta-cel and Voxzogo, while molnupiravir heads for an advisory panel.

Johnson & Johnson's ciltacabtagene autoleucel could soon become the second approved cell therapy for multiple myeloma, with its Pdufa date falling towards the end of next month. The BCMA-targeted Car-T therapy is gunning to take on Bristol Myers Squibb/Bluebird's Abecma, and boasts better efficacy.

Elsewhere, Biomarin is taking its achondroplasia project back to the regulators after submitting durability data, while a panel will decide whether molnupiravir, Merck & Co/Ridgeback’s oral Covid-19 antiviral, has safety in hand.

Second place, but for how long?

Cilta-cel’s filing was based on Cartitude-1, an open-label study in relapsed/refractory multiple myeloma patients who had received at least three prior lines of therapy or were double-refractory to a proteasome inhibitor and immunomodulatory drug.

Efficacy reported at this year’s Asco was impressive, with the overall response rate in 97 patients hitting 98%, including an 80% complete response rate and median progression-free survival of nearly 23 months.

Bristol Myers Squibb/Bluebird's Abecma, which was approved for fifth-line use back in March, cites 72% ORR and a 28% complete response rate on its label.

Abecma’s label also includes a black box warning of haemophagocytic lymphohistiocytosis/macrophage activation syndrome and cytopenia, in addition to the cytokine release and neurotoxicities that are standard with Car-T therapies. Cilta-cel will probably contain a similar warning, and has been associated with higher neurotoxicity than Abecma.

Greater opportunity lies in earlier lines of therapy, but toxicity could hold up both Abecma and cilta-cel.

Durability still a question

In August Biomarin’s vosoritide, now called Voxzogo, became the first medicine approved in Europe for the treatment of children aged two and over with achondroplasia. A decision in the US is due in November after a three-month delay.

Biomarin’s original NDA filing included one-year phase 3 data in children over five years old given daily

injections of Voxzogo. The FDA, however, expressed a desire for two-year controlled data to ensure that the efficacy of Voxzogo was durable. In Europe one-year data had been enough for approval.

Additional data from the open-label extension study were subsequently submitted to the US regulator, though this did not include a direct placebo-controlled comparison. Durability was shown through a comparative analyses of participants randomised to the active arm versus participants on placebo with two years' untreated follow-up.

Voxzogo is forecast to become the top-selling achondroplasia project in 2026, according to *Evaluate Pharma* consensus. Ascendis's Transcon CNP, the most advanced rival, is still only in phase 2. But Ascendis is going for convenience, testing once-weekly treatment, with an update from two placebo-controlled trials due this quarter.

Molnupiravir's panel

Safety will likely dominate the discussion at a November 30 US adcom for Merck & Co and Ridgeback's molnupiravir. The oral antiviral yielded impressive results last month in Covid-19 outpatients, but some concern has been voiced over its potential for mutagenicity.

Molnupiravir works by causing a large number of genetic mutations in the replicating virus, and the worry is that mutations might also occur in the RNA of patients who take it, though Merck insists that it has not found anything worrying.

If given backing, and a subsequent approval, molnupiravir offers convenience advantages versus infused antibodies. Another phase 3 study, Move-Ahead, in post-exposure prophylaxis is due to report next year.

The tables below list first-time and supplementary US approvals, as well as advisory committee meetings due next month, with consensus forecasts from *Evaluate Pharma*.

Notable first-time US approval decisions due in November

Project	Company	PDUFA date	Indication(s)	2026e sales by indication ($m)	Note
Doria (risperidone ISM)	Laboratorios Farmacéuticos Rovi	Est Nov	Schizophrenia	376	Long-acting injectable
OTL38 (pafolacianine sodium injection)	On Target Laboratories	Est Nov 3	Adjunct for identifying ovarian cancer during surgery	–	Binds to folate receptors and illuminates intraoperatively under near-infrared light
Topiramate oral solution (ET-101)	Azurity/ Eton	Nov 6	Tonic-clonic seizures, partial-onset seizures, and as preventative treatment of migraine	–	Extended from Aug
LIQ861 (treprostinil inhalation powder)	Liquidia	Nov 7 (resubmission)	Pulmonary arterial hypertension	126	Previous CRL for CMC issues
Besremi (ropeginterferon alfa-2b)	Pharmaessentia	Nov 13 (resubmission)	Polycythemia vera	–	Previous CRL due to manufacturing inspection delay
Maribavir	Takeda	Est Nov 19	Post-transplant cytomegalovirus infection in refractory pts with/without resistance	354	Backed by adcom in October

Notable first-time US approval decisions due in November					
Voxzogo (vosoritide)	Biomarin	Nov 20	Achondroplasia	720	See text
Fyarro (ABI-009)	Aadi Bioscience	Nov 26	Advanced malignant PEComa	–	Nab-sirolimus
Pedmark	Fennec	Nov 27 (resubmission)	Cisplatin-induced ototoxicity for paediatric patients	165	Previous CRL (CMC)
Ciltacabtagene autoleucel (cilta-cel)	J&J/ Legend Biotech	Nov 29	3L+ multiple myeloma	1,692	See text
Enpaxiq (pacritinib)	CTI Biopharma	Nov 30	Myelofibrosis patients with severe thrombocytopenia	368	Oral kinase inhibitor with specificity for Jak2, Irak1, and CSF1R
Plinabulin + G-CSF	Beyondspring	Nov 30	Prevention of chemotherapy-induced neutropenia	138	Also being tested in NSCLC, but data were unimpressive (*Esmo 2021 – Beyondspring experiences winter of discontent*)
Sci-B-Vac	Opko Health/ VBI Vaccines	Nov 30	Hepatitis B	164	3-antigen hepatitis B vaccine
Oleogel-S10 (Filsuvez)	Amryt	Nov 30	Epidermolysis bullosa	285	Could become the first approved treatment (*Epidermolysis bullosa gene therapies wait in the wings*)
Cibinqo (abrocitinib)	Pfizer	Q4?	Atopic dermatitis	1,035	Timings to be confirmed; with the FDA's recent safety review of the class approval could include suboptimal dosing and black box warnings
Epsolay	Sol-Gel	Q4?	Papulopustular rosacea	–	Delayed in April as Covid-19 travel restrictions prevented pre-approval inspection
Eohilia (TAK-721)	Takeda	Q4?	Eosinophilic esophagitis	187	April Pdufa missed

CMC=Chemistry, manufacturing and controls. Source: Evaluate Pharma & company releases.

Advisory committee meetings in November					
Project	**Company**	**Adcom date**	**Indication**	**2026e sales by indication ($m)**	**Note**
LV-101 (intranasal carbetocin)	Levo	Nov 4	Hyperphagia, anxiety, and distress behaviours associated with Prader-Willi syndrome	–	Pdufa expected by YE
Molnupiravir	Merck/ Ridgeback	Nov 30	EUA for the treatment of mild to moderate Covid-19 in adults who are at risk for progressing to severe Covid-19 and/or hospitalisation	–	See text
Source: FDA adcom calendar & Evaluate Pharma.					

Supplementary and other notable approval decisions in November			
Product	**Company**	**Indication (clinical trial)**	**Date**
BAT1706 (Avastin biosimilar)	Bio-Thera Solutions	Colorectal cancer (+ chemo), 1L non-squamous NSCLC, recurrent glioblastoma, renal cell carcinoma (+ interferon alfa), cervical cancer	Nov 27
Xeljanz	Pfizer	Ankylosing spondylitis (A3921120)	Q4?
Olumiant	Lilly	Atopic dermatitis (Breeze-AD programme)	Q4?
Rinvoq	Abbvie	Atopic dermatitis, psoriatic arthritis and ankylosing spondylitis	Q4?
Source: Evaluate Pharma & company releases.			