Evaluate Vantage

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US FDA approval tracker: a first for Aimmune

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January saw Aimmune receive an FDA approval for Palforzia, a first for peanut allergy, alongside decisions for Blueprint Medicines and Horizon Therapeutics.

The first month of 2020 saw the US FDA approve the first peanut allergy treatment, Aimmune's Palforzia, greenlit after markets closed on Friday. The company's shares opened up 17% today, but quickly retreated to a 4% gain on concerns about Palforzia's commercial opportunity.

The treatment has a black box warning for anaphylaxis and a restrictive risk-mitigation programme as it is based on a tolerising approach to peanut by the delivery of very small amounts of peanut powder in a capsule to the patient.

Questions remain over the long term benefits versus peanut avoidance, and in Palforzia's clinical program patients on treatment had greater Epipen usage than those not on treatment, a consideration for payers given that this will come on top of the ~$10k annual cost of Palforzia.

EvaluatePharma's consensus puts 2024 sales at $1.3bn, a forecast that comprises some very different opinions; Stifel analysts, who are among the most conservative, believe that Palforzia will be become a "niche therapy". On the competitor front DBV's desensitisation patch Viaskin Peanut has a PDUFA date in August.

Early

Other positive decisions from the FDA last month included Blueprint's Ayvakit, a month before its designated PDUFA date. Ayvakit is now approved for treating gastrointestinal stromal tumours driven by the PDGFRα exon 18 mutation, including D842V mutations. Expanding Ayvakit into a fourth-line setting will have to wait until the Voyager study reads out, due in the second quarter.

The decision on Horizon's Tepezza was not expected until March, but the antibody became the first approved treatment for thyroid eye disease last month. The autoimmune condition leads to eye bulging and sometimes blindness. The disease can occur on its own but is also present in up to half of people with Graves' disease.

The label language was broader than expected with no restrictions around disease type or severity, a topic that was heavily discussed at the unanimous ad com at the end of last year. The treatment has an annual list price of $200k and Horizon has guided to 2020 sales of $30-40m. 2024 sales are forecast to reach $484m according to *EvaluatePharma* consensus.

Notable first-time US approval decisions in January			
Project	**Company**	**2024e sales ($m)**	**Outcomes**
Palfozia (AR101)	Aimmune	1,312	Approved
Rimegepant ODT/Zydis ODT	Biohaven	885 (rimegepant franchise)	No decision yet, guided as Q1
Ayvakit (avapritinib)*	Blueprint Medicines	673	Approved early
Tazverik (tazemetostat)	Epizyme	556	Approved
Tepezza (teprotumumab)*	Horizon Therapeutics	484	Approved early
Dificid (oral suspension)	Merck/Astellas	168 (tablet and suspension)	Approved
Travivo	Fabre-Kramer Pharmaceuticals	-	No decision yet
*Source: EvaluatePharma. *Not in January PDUFA story, source Go or no go? Enhertu's destiny revealed and FDA decisions due for Epizyme, Novo, December 23, 2019.*			

And the rest

Novo Nordisk's semaglutide was up for two decisions last month but only Ozempic, the once-weekly version, got its cardiovascular risk-reduction claim. Rybelsus, the oral, once-daily version, managed to get Pioneer 6 data added to its label, but that only showed non-inferiority to placebo. Rybelsus will have to wait for data from a long-term outcome study, Soul, which began earlier this year.

A decision on Biohaven's Zydis ODT, a fast-acting dissolving tablet formulation of rimegepant for the acute treatment of migraine, is also still pending. More importantly the results of a phase III prevention study with the tablet formulation are expected soon, and could position rimegepant as a more convenient alternative to approved injectable CGRP inhibitors.

Supplementary and other notable approval decisions in January			
Product	**Company**	**Event type**	**Outcomes**
Keytruda	Merck & Co	sBLA for high risk non-muscle invasive bladder cancer, with carcinoma in situ unresponsive to standard BCG therapy, based on Keynote-057	Approved
Ozempic and Rybelsus (oral semaglutide)	Novo Nordisk	sNDA for cardiovascular risk reduction	Approved for Ozempic (Sustain 6; Soul data needed for Rybelsus claim)
Dificid (tablet)	Merck & Co/Astellas	sNDA for *Clostridium difficile* infections in children aged six months or older, based on Sushine study	Approved
Imfinzi	Astrazeneca	sBLA for first-line SCLC, based on Caspian trial	No decision yet, guided as Q1
Mycamine	Astellas	sNDA for invasive candidiasis in paediatric patients under 4 months of age	Approved
Source: Go or no go? Enhertu's destiny revealed and FDA decisions due for Epizyme, Novo, December 23, 2019.			

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