Evaluate Vantage

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US FDA approval tracker: April

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Last month the US regulator tightened its grip over PI3K inhibitors by calling for randomised data instead of single-arm clinical studies. Ahead of an advisory committee meeting on the subject, TG Therapeutics pulled its own PI3K asset Ukoniq from sale and withdrew the filing of its combination product, U2. Elsewhere both Alnylam and Merck had Pdufa dates extended. For the latter, its 15-valent pneumococcal vaccine Vaxneuvance was heading for a decision in infants and children, a larger market than its already approved adult label. Despite the three-month delay Merck is still ahead of Pfizer in the younger age group, with that group's follow-on project, Prevnar 20, due to yield phase 3 in the second half of the year. Lastly Bristol Myers Squibb finally received the green light for mavacamten, now called Camzyos, in obstructive hypertrophic cardiomyopathy. The cardiac myosin inhibitor not only comes with a REMS and a black boxed warning, but a higher than expected price tag. With a list price of $89,500 a year it is well above the $15,000 figure ICER said would meet traditional thresholds of cost-effectiveness. Bristol has its work cut out to meet its $4bn-plus peak sales target, and justifying the $13.1bn it spent on Myokardia.

Notable first-time US approval decisions in April				
Project	**Company**	**Indication(s)**	**2026e sales by indication (SBI) ($m)**	**Outcome**
Vutrisiran	Alnylam	Polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis	1,828*	Delayed to July 14 (review of new information related to the secondary packaging and labelling facility)
Camzyos (mavacamten)	Bristol Myers Squibb	Obstructive hypertrophic cardiomyopathy	1,672	Approved
Bimzelx	UCB	Plaque psoriasis	953	Pending (H1)
Igalmi (dexmedetomidine/ BXCL501)	Bioxcel	Agitation associated with schizophrenia and bipolar disorders I and II	378	Approved
Sulanda (surufatinib)	Hutchmed	Pancreatic and non-pancreatic neuroendocrine tumors	362	CRL (multi-regional clinical trial needed)
FT218	Avadel	Narcolepsy	321	Pending
AXS-07	Axsome	Acute treatment of migraine	128	CRL (CMC issues)
Epsolay cream	Sol-Gel/ Galderma	Papulopustular rosacea	-	Approved
Vivjoa (oteseconazole)	Mycovia	Recurrent vulvovaginal candidiasis	-	Approved
Cuvrior (Cuprior)	Orphalan	Adult patients with stable Wilson’s disease	-	Approved
Tuoyi (toripalimab)	Coherus/ Shanghai Junshi	1st-line chemo combo & 3rd-line monoRx nasopharyngeal carcinoma	-	CRL (quality process change required)
Tapinarof	Dermavant	Plaque psoriasis	-	Pending (Q2)
Annik (penpulimab)	Akeso/Sino	3L nasopharyngeal carcinoma	-	Pending (H1)
SH-111	Shorla Oncology (private)	T-cell leukaemia	-	Pending
**SBI as amyloidosis. Source: company statements, Evaluate Pharma*				

Advisory committee meetings in April			
Project	**Company**	**Indication**	**Outcome**
Covid-19 vaccination	N/A	Covid-19 prevention	Agency will reconvene its booster discussion on June 28
PI3K inhibitor class	N/A	Haematologic malignancies	16-0, future approvals of PI3K inhibitors should be supported by randomized data
U2, Ukoniq	TG Therapeutics	Haematologic malignancies	Prior to ad com U2 application withdrawn and Ukoniq pulled
Source: FDA ad com calendar, Evaluate Pharma			

Supplementary and other notable approval decisions in April			
Product	**Company**	**Indication (clinical trial)**	**Outcome**
Vaxneuvance	Merck	Prevention of invasive pneumococcal disease in children 6 weeks through 17 years of age (15-valent conjugate vaccine, PNEU-PED)	Delayed to July 1 (additional analyses of data from the paediatric studies)
REgen-Cov (Ronapreve)	Regeneron	Treat Covid-19 in non-hospitalised patients and as prophylaxis in certain individuals	Delayed to July 13 (already has EUA)
Yescarta	Gilead	2L relapsed/refractory large B-cell lymphoma (Zuma-7)	Approved
Qelbree	Supernus	ADHD in adults (P306)	Approved
Ultomiris	Astrazeneca	Adults with generalised myasthenia gravis who are anti-acetylcholine receptor antibody-positive (NCT03920293)	Approved
Rinvoq	Abbvie	Ankylosing spondylitis (Select-Axis 2, Select-Axis 1)	Approved
Vijoice (alpelisib, marketed as Piqray in breast cancer)	Novartis	Adult and paediatric patients 2 years of age and older with PIK3CA-related overgrowth spectrum (EPIK-P1)	Approved
Cysteine hydrochloride (Elcys generic)	Eton	Additive to amino acid solutions to meet the nutritional requirements of newborn infants	Approved
Alymsys (Avastin biosimilar)	Amneal/ mAbxience	Metastatic colorectal cancer, 1L NSCLC, recurrent glioblastoma, met renal cancer, met cervical cancer, epithelial ovarian, fallopian tube, or primary peritoneal cancer	Approved
Veklury	Gilead	Paediatric patients under 12 years old hospitalised with Covid-19 or have mild-to-moderate Covid-19 at high risk of progression to severe Covid-19	Approved
Caplyta	Intra-cellular	New dosage strengths for schizophrenia and bipolar depression	Approved
Olumiant	Lilly	Atopic dermatitis (Breeze-AD programme)	CRL
TV-46000/mdc-IRM (risperidone extended-release)	Teva/ MedinCell	Schizophrenia (Rise, Shine)	CRL
Enhertu	Astra/Daiichi	Unresectable/metastatic Her2 +ve breast cancer pts who have received a prior anti-Her2-based regimen (Destiny-Breast 03)	Pending (Q2)
Olumiant	Lilly	Treatment of certain hospitalised patients with Covid-19	Pending (Q2)
Kymriah	Novartis	r/r FL after two prior lines of treatment (Ph2 Elara)	Pending
Source: company statements, Evaluate Pharma			

FDA Covid-19 EUAs		
Product	**Company**	**Outcome**
Xevudy (sotrovimab)	GSK/Vir	No longer authorised to treat Covid-19 in any US region as Xevudy is unlikely to be effective against the BA.2 sub-variant (gained EUA in May 2021)
Source: company releases		