Evaluate Vantage

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# US FDA approval tracker: August

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Fibrogen’s roxadustat received an expected FDA knockback last month after a negative panel in July. The FDA requested another study, but in the EU the outcome was wildly different, and full approval was granted eight days after the US disappointment. Two other US complete response letters were handed out, one for Spectrum’s Rolontis, owing to manufacturing issues, and the other for Sesen’s Vicineum. The regulators requested a whole new confirmatory trial for Sesen’s bladder cancer project, pushing a resubmission out to 2023. However, it was not all bad news last month as several approval decisions pegged for September came early. The most notable involved Ascendis’s growth hormone Skytofa, and Bristol Myers Squibb’s Opdivo for adjuvant treatment of high-risk urothelial carcinoma; the latter had been filed for muscle-invasive disease, based on the Checkmate-274 study, but the approved label makes no mention of this stipulation *(Go or no go? A first for Nefecon, August 27, 2021).*

| Notable first-time US approval decisions in August | | | | |
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| **Project** | **Company** | **Indication(s)** | **2026e sales by indication ($m)** | **Outcome** |
| Comirnaty | Pfizer/Biontech | Prevention of Covid-19 (aged 16 and older) | 2,819 | Full approval (still under EUA for ages 12-15) |
| Evrenzo (roxadustat) | Fibrogen/Astrazeneca/ Astellas | Anaemia in patients with chronic kidney disease on/not on dialysis | 1,666 | CRL (additional trial needed) |
| Skytrofa (Transcon hGH/ lonapegsomatropin) | Ascendis | Paediatric growth hormone deficiency | 1,371 | Approved (~1mth early) |
| AXS-05 | Axsome | Major depressive disorder | 1,277* | Delayed (no new date given) |
| Welireg (MK-6482/belzutifan) | Merck & Co | Von Hippel-Lindau disease-associated renal cancer | 386 | Approved (~1mth early) |
| Nexviazyme (avalglucosidase alfa) | Sanofi | Pompe disease | 366 | Approved |
| Korsuva injection | Cara/Vifor | Pruritis in haemodialysis patients | 358 | Approved |
| Rolontis | Spectrum | Neutropenia in patients receiving myelosuppressive anti-cancer drugs | 330 | CRL (manufacturing, reinspection needed) |
| Vicineum | Sesen Bio | High risk BCG-unresponsive non-muscle invasive bladder cancer | 292 | CRL (CMC & new trial) |
| TicoVac | Pfizer | Tick-borne encephalitis | 218 | Approved |
| Topiramate oral solution | Eton | Tonic-clonic seizures, partial-onset seizures, and as preventative treatment of migraine | - | Extended to Nov 6 |
| Epsolay | Sol-Gel | Papulopustular rosacea | - | No decision yet |

**SBI not split out by depression type. Source: Evaluate Pharma & company releases.*

| Supplementary and other notable approval decisions in August | | | |
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| **Product** | **Company** | **Indication (clinical trial)** | **Outcome** |
| Jemperli | GSK/Anaptysbio | Adult patients with MMR-deficient recurrent or advanced solid tumours (Garnet) | Approved (accelerated) |
| Jardiance | Lilly/Boehringer | Heart failure with reduced ejection fraction (Emperor-Reduced) | Approved (~3wks early) |
| Opdivo | Bristol Myers Squibb | Adjuvant treatment for patients with high-risk urothelial carcinoma (Checkmate-274) | Approved (2wks early) |
| Xarelto | J&J | Peripheral artery disease patients post lower-extremity revascularisation (Voyager PAD) | Approved |
| Tibsovo | Servier | Previously treated, locally advanced or metastatic cholangiocarcinoma with an IDH1 mutation (ClarIDHy) | Approved |
| Xywav | Jazz | Adult patients with idiopathic hypersomnia (NCT03533114) | Approved |
| Keytruda + Lenvima | Merck/Eisai | 1L renal cell carcinoma (Clear/Keynote-581/Study 307) | Approved |
| *Source: Evaluate Pharma & company releases.* | | | |

| Voluntarily withdrawn accelerated approvals | | |
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| **Product** | **Company** | **Withdrawn indication (clinical trials)** |
| Istodax | Bristol Myers Squibb | 2L peripheral T-cell lymphoma |
| Tecentriq (+ Abraxane) | Roche | PD-L1-positive, 1L triple-negative breast cancer (Impassion-130, Impassion-131) |
| *Source: company releases.* | | |