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November US FDA User Fee Calendar: Takeda On Tap; Small Molecules For Cancer

by Bridget Silverman

Takeda hopes for good news on fruquintinib and TAK-755, while Bristol and SpringWorks share a goal date for their breakthrough-designated cancer treatments.

Takeda Pharmaceutical Co. Ltd. is looking forward to US FDA action on two novel agents during the month of November. If they are both approvals, they could provide welcome new storylines after the company pulled the BLA for its dengue vaccine Qdenga in July and decided to withdraw targeted lung cancer therapy Exkivity (mobocertinib) from the market last month following failed in a Phase III confirmatory trial.

Takeda's TAK-755 – an enzyme replacement therapy combining apadamtase alfa and cinaxadamtase alfa – is first up with a 16 November 2023 user fee goal date for congenital thrombotic thrombocytopenic purpura (cTTP). TAK-755's rare pediatric disease designation puts Takeda in line for a priority review voucher if FDA approves the product.

Takeda and *HUTCHMED (China) Limited*'s kinase inhibitor fruquintinib could close out the month thanks to its 30 November PDUFA goal for previously treated metastatic colorectal cancer.

TAK-755 and fruquintinib are joined by six other novel agents with November 2023 Prescription Drug User Fee Act goal dates, according to the Pink Sheet FDA Performance Tracker's *User Fee Goal Dates* chart. (*See table at end of story.*)

Fewer applications for new uses of approved medicines are on the November calendar, lead by pediatric indications for *Boehringer Ingelheim GmbH* and *Eli Lilly and Company*'s Jardiance (empagliflozin) in type 2 diabetes and Boehringer Ingelheim's Ofev (nintedanib) in fibrosing interstitial lung disease.

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Cancer At Center Stage

Four of the eight novel agents with upcoming goal dates are seeking oncology indications. That's a higher concentration of oncologics than for FDA's 2023 approvals to date: cancer treatments make up 20% of the Center for Drug Evaluation and Research's new molecular entity and novel biologic approvals this year.

27 November holds PDUFA goals for two of the oncologics, both of which carry breakthrough therapy designations: *Bristol Myers Squibb Company*'s next-generation tyrosine kinase inhibitor repotrectinib and *SpringWorks Therapeutics Inc.*'s gamma secretase inhibitor nirogacestat.

Bristol is seeking an indication for ROS-1 positive non-small cell lung cancer for repotrectinib, which had been a centerpiece of Bristol's acquisition of *Turning Point Therapeutics Inc*. The company continues to pursue growth through acquisitions in the targeted therapy space, recently announcing plans to acquire *Mirati Therapeutics, Inc.* and its KRAS G12C inhibitor Krazati (adagrasib) for KRAS-mutated NSCLC. (Also see "*Bristol's New Launches Hit Reality As New Product Portfolio Forecast Is Revised*" - Scrip, 26 Oct, 2023.)

SpringWorks Therapeutics' NDA for nirogacestat to treat desmoid tumors is being considered under the FDA's Real-Time Oncology Review (RTOR) pilot program. The drug originally had an August goal date, but it was extended by three months so FDA could review additional analyses of previously submitted data, meaning nirogacestat will not receive one of the very fast approvals that the RTOR program sometimes produces. (Also see "*A 3-Month Delay For A Better Label: US FDA User Fee Goal Extensions Are Usually Good News*" - Pink Sheet, 28 Aug, 2023.)

Lumicell Inc.'s fluorescent imaging agent Lumisight (pegulicianine) cannot claim a breakthrough therapy designation for itself, but the Lumicell Direct Visualization System that the drug is used with holds a breakthrough device designation. Lumisight is designed to aid in detection of residual breast cancer tissue during surgery.

Catheters And Chikungunya

Valneva USA Inc. is hoping to receive the FDA's first approval for a vaccine for chikungunya disease when its single-dose vaccine VLA1553 reaches its mid-November goal date, when it could earn a tropical disease priority review voucher. The BLA seeks accelerated approval, which would be a first for an outbreak disease, the company noted; the original August goal date was extended to support alignment and agreement on the Phase IV program.

VLA1553 is the only non-oncologic with a breakthrough therapy designation on the November user fee calendar.

A different approach to infectious disease prevention also appears on the November calendar, in the form of *CorMedix Inc.*'s Defencath (taurolidine, heparin and citrate), a non-antibiotic

antimicrobial and antifungal combination for use as a catheter lock solution to prevent bloodstream infections associated with the use of central venous catheters in patients undergoing chronic hemodialysis.

The Defencath NDA has suffered from compliance deficiencies at a contract manufacturer and the active pharmaceutical ingredient heparin supplier that spurred two complete response letters from the FDA. For the third review cycle, CorMedix established an alternative contract manufacturer and an alternative source of heparin API. The FDA is slated to decide on Defencath by its 15 November goal date.

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