Japan to Set Up Dedicated Team to Expedite Review of Sakigake-Designated Cell/Gene Therapies

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Japan's Pharmaceuticals and Medical Devices Agency (PMDA) will establish a team dedicated to reviewing cell and gene therapies designated for its *sakigake* fast-track pathway in FY2020 as it gears up for a flurry of approvals for these up-and-coming products.

Funds necessary for this plan is expected to be included in the Ministry of Health, Labor and Welfare's (MHLW) budget request for FY2020, which begins next April. The specific amount being sought will be announced early next week as the MHLW is mandated to submit its request to the Ministry of Finance by the end of August, which will be screened towards the end of the year.

The *sakigake* fast-track review system, which was introduced on a pilot basis in 2015, covers drugs and medical devices as well as "regenerative medicine products," which include cell and gene therapies. The system - comparable with the US/Europe's Breakthrough Therapy/PRIME programs - is designed to facilitate the development and approval of innovative products in Japan ahead of the rest of the world, targeting a shortened review timeline of just six months.

To enhance the system, the MHLW in FY2019 secured budgets for adding a so-called "concierge" review partner, who keeps tabs on the progress of the designated products' development and reviews, as well as two review teams. However, these were for the review of drugs and medical device products.

Under the system, a total of 11 "regenerative medicine products" have been designated between 2016 and 2019, with two to three selected in each of the past four designation rounds, which took place in February 2016, February 2017, March 2018, and April 2019.

One of these products, Sapporo Medical University/Nipro's Stemirac, a stem cell therapy for spinal cord injury, was granted conditional approval in December. Novartis' Zolgensma (onasemnogene abeparvovec-xioi), a gene therapy for spinal muscular atrophy (SMA), was filed in Japan in November and is pending approval.

4 Submissions in FY2019?

Following these, four products could reach the regulatory filing stage as early as FY2019: 1) University of Tokyo/Daiichi Sankyo's oncolytic virus G47Δ (recombinant herpes virus; glioma), 2) SanBio's stem cell therapy

SB623 (traumatic brain injury), 3) Takara Bio/Otsuka Pharmaceutical's gene therapy TBI-1301 (NY-ESO-1 antigen-specific TCR-gene-transduced autologous T lymphocytes; synovial sarcoma), and 4) Tokyo Women's Medical University/CellSeed's oral mucosa-derived esophageal cell sheet CLS2702C/D (esophageal cancer).

The MHLW plans to continue bestowing the *sakigake* status to further regenerative medicine products, with the number expected to increase further down the road. It is expected to invite applications for the fifth round of designations as early as this autumn, according to a typical timeline.

If the budget for the dedicated review team goes through, it would help the PMDA accelerate its review of *sakigake*-designated cell and gene therapies, making their early commercialization possible, and also help boost the number of designations.