April 25, 2019


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# SanBio Announces SB623, an Investigational Product, Granted Advanced Therapy Medicinal Product Classification by European Medicines Agency

SanBio Co., Ltd. announces today that SB623, an Investigational Product, Granted Advanced Therapy Medicinal Product Classification by European Medicines Agency as attached.

The SanBio Group (SanBio Co., Ltd. and SanBio, Inc.) is aiming to submit an application for manufacturing and marketing approval for its TBI program in Japan during the fiscal year ending January 31, 2020 (February 2019–January 2020), using the conditional and term-limited authorization system for regenerative medicine products under the Revised Pharmaceutical Affairs Act of Japan. The company also plans to initiate a Phase 3 trial for SB623 for the treatment of chronic motor deficit from TBI by the end of fiscal year ending January 31, 2020. This Advanced Therapy Medicinal Product (ATMP) classification will advance the clinical development of SB623 in Europe.

The impact of this event on the Company's financial results for the fiscal year ending January 31, 2020, will be examined. If any matters need to be disclosed, the Company will announce them promptly.

# SB623, an Investigational Product, Granted Advanced Therapy Medicinal Product Classification by European Medicines Agency

**Mountain View, Calif.—April 25, 2019—** The SanBio Group (SanBio Co., Ltd. and SanBio, Inc.), a scientific leader in regenerative medicine for neurological disorders, today announced that Advanced Therapy Medicinal Product (ATMP) classification has been granted for SB623 for the treatment of patients with motor deficits arising from Acquired Brain Injury, including traumatic brain injury, ischaemic stroke and haemorrhagic stroke.

Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells. Determined by the Committee for Advanced Therapies (CAT) at the European Medicines Agency (EMA), treatments that receive ATMP classification may offer groundbreaking new opportunities for the treatment of disease and injury.[i]

SB623 is an investigational product made from modified and cultured adult bone marrow-derived mesenchymal stem cells that undergo temporary genetic modification. Implantation of SB623 cells into injured nerve tissue in the brain is expected to trigger the brain's natural regenerative ability to recover lost motor functions.

"Traumatic brain injury is among the most common health conditions faced worldwide, with more than 57,000 TBI-related deaths and 1.5 million TBI-related hospital discharges occurring each year in Europe,[ii]" said Keita Mori, chief executive officer at SanBio. "We hope this ATMP classification allows us the opportunity to work closely with European regulators around the development and regulatory pathway for SB623."

On April 16, SanBio presented positive Phase 2 results from the STEMTRA trial at the American Association of Neurological Surgeons (AANS) annual scientific meeting. In this clinical study involving a total of 61 patients, 46 were treated with SB623 and 15 underwent sham surgery as a control group. Improvement was measured by the change from baseline in the Fugl-Meyer Motor Scale (FMMS) score. This scale measures changes in motor impairment and a 10 or more point improvement has been considered a clinically meaningful threshold in the context of acquired brain injury.[1]

In this study, SB623 met its primary endpoint, with patients treated with SB623 achieving an average 8.7 point improvement from baseline in the FMMS, versus 2.4 in the control group, at 24 weeks (p=0.044). Of patients treated with SB623, 18 (39.1%) reached this threshold compared to one control patient (6.7%; p=0.04). No new safety signals were identified. The most commonly reported adverse event were headaches. There were no significant differences in the rate of adverse events between patients treated with SB623 and placebo.

SanBio plans to initiate a Phase 3 trial for SB623 for the treatment of chronic motor deficit from TBI by the end of fiscal year ending January 31, 2020. The company is also aiming to submit an application for manufacturing and marketing approval for its TBI program in Japan during the fiscal year ending January 31, 2020 (February 2019–January 2020), using the conditional and term-limited authorization system for regenerative medicine products under the Revised Pharmaceutical Affairs Act of Japan.

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[1] Feys HM et al., 1998; van der Lee JH, et al., 2001

**About the STEMTRA Trial**
STEMTRA is a 12-month, Phase 2, randomized, double-blind, surgical sham-controlled, global trial evaluating the efficacy and safety of SB623 compared to sham surgery in patients with stable chronic motor deficits secondary to traumatic brain injury. In this study, SB623 cells were implanted directly around the site of brain injury.

To be eligible for this trial, patients (ages 18-75) must have been at least 12 months post-TBI and had a Glasgow Outcome Scale extended (GOS-E) score of 3-6 (e.g., moderate or severe disability). Patients must also have been able to undergo all planned neurological assessments and had no seizures in prior three months. The primary endpoint was mean change from baseline in Fugl-Meyer Motor Scale (FMMS) score at six months. The STEMTRA trial enrolled 61 patients from 13 surgical and 18 assessment sites in the U.S., Japan and Ukraine.

**About SanBio, Inc.**
SanBio is a regenerative medicine company headquartered in Tokyo and Mountain View, California, with cell-based products in various stages of research, development and clinical trials. Its proprietary cell-based investigational product, SB623, is currently in a Phase 2b clinical trial for treatment of chronic motor deficit resulting from ischemic stroke, and in a Phase 2 clinical trial for treatment of chronic motor deficit resulting from traumatic brain injury. More information about SanBio, Inc. is available at http://sanbio.com.

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**For more information, contact:**

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[i] Advanced therapy medicinal products: overview. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/overview/advanced-therapy-medicinal-products-overview Last accessed April 17, 2019.

[ii] Majdan M, Plancikova D, et al. Years of life lost due to traumatic brain injury in Europe: A cross-sectional analysis of 16 countries. *PLOS Medicine*. 2017;14(7).