AANS 2019: SB623 Demonstrated Statistically Significant Improvement in Motor Function Among Patients with Chronic Motor Deficit from Traumatic Brain Injury (TBI)

Positive results from the STEMTRA Phase 2 trial were presented today at the American Association of Neurological Surgeons (AANS) annual scientific meeting

Data demonstrates the possibility of a stem cell treatment for chronic motor deficit from TBI

Mountain View, Calif.—April 16, 2019— The SanBio Group (SanBio Co., Ltd. and SanBio, Inc.), a scientific leader in regenerative medicine for neurological disorders, today announced that 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, in a Phase 2 trial of patients with chronic motor deficit from traumatic brain injury (TBI). Detailed results from the STEMTRA Phase 2 trial evaluating the efficacy and safety of SB623 were presented today at the American Association of Neurological Surgeons (AANS) annual scientific meeting in San Diego.

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.

According to the Centers for Disease Control (CDC), approximately 5.3 million people in the United States are living with a TBI-related disability and more than 280,000 people annually suffer permanent disability.[i] The effects of TBI are often long-lasting, with more than one-third of severe TBI patients displaying a neuromotor abnormality two years following an injury.[ii]

"Traumatic brain injury is among the most common health conditions faced worldwide, yet its devastating and long-lasting effects are often overlooked and underestimated," said Dr. Okonkwo, M.D., Ph.D., professor of neurological surgery and director of the Neurotrauma Clinical Trials Center (NCTC) at the University of Pittsburgh. "The results of this study are truly groundbreaking, demonstrating the possibility of regenerating the brain following injury—a finding that could have significant implications for research in traumatic brain injury and other brain diseases."

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] Of patients treated with SB623 , 18 (39.1%) reached this threshold compared to one control patient (6.7%). This difference was statistically significant (p=0.044).

No new safety signals were identified. The most commonly reported adverse event were headaches and 34.4%of patients treated with SB623 had headache up to 7 days after surgery. There were no significant differences in the rate of adverse events between patients treated with SB623 and placebo (p=0.25).

---

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

“These data are the first in the world to demonstrate the possibility of a stem cell treatment that may regenerate brain cells following traumatic brain injury, which affects thousands of people each year and has no underlying cure,” said Keita Mori, chief executive officer at SanBio. “These results represent a significant step forward in the development of regenerative medicines for neurological disorders, and we are thrilled to be able to share these findings at the largest gathering of brain surgeons in the world.”

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.

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.

# # #

For more information, contact:
SanBio Co., Ltd.
Management Administration
info@sanbio.jp

---

[i] Centers for Disease Control and Prevention. Report to Congress on Traumatic Brain Injury in the United States: Epidemiology and Rehabilitation. National Center for Injury Prevention and Control; Division of Unintentional Injury Prevention. Atlanta, GA. 2014.

[ii] Walker WC, Pickett TC. Motor impairment after severe traumatic brain injury: a longitudinal multicenter study. *Journal of Rehabilitation Research & Development*. 2007;44(7):975-982.