アナリスト・機関投資家向け説明会資料
「慢性期外傷性脳損傷プログラム第2相試験（STEMTRA 試験）の最終解析結果について」

サンバイオ 株式会社

東証グロース：4592

2022年4月13日

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1. 学会発表サマリー

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STEMTRA試験 最終解析結果を学会発表

米国神経学会 2022年次総会 Clinical Trials Plenary Sessionにて発表

開催日時：4月2～7日

開催場所：米国ワシントン州シアトル

発表者　：Dr. Peter McAllister

(ニューイングランド神経・頭痛研究所の医長兼チーフ・メディカル・オフィサー)

発表内容： STEMTRA試験の48週　有効性、安全性データについて

＜STEMTRA試験＞

- 日米グローバルで行ったフェーズ2臨床試験。外傷性脳損傷に伴う慢性期運動機能障害に対するSB623の脳内投与の有効性と安全性を評価した無作為化、二重盲検、偽手術対照、多施設、国際共同フェーズ2臨床試験
- SB623投与後24週時点でのベースラインからの運動機能障害の改善（主要評価項目）において、対象群と比較して統計的に有意な改善を示した（Neurology 2021）

※米国神経学会（American Academy of Neurology: AAN）・・・38,000人以上の神経科医および神経科学の専門家が所属する世界有数の学会

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学会発表の反響

発表内容が多数のメディアに掲載

患者ベネフィットに関するDr. Peter McAllisterのコメント

"Patients able to use their hands that they could not use, patients never spoke a words, with aphasic after his TBI, say his first words“ (NeurologyLive)

"Some who couldn't move their arm at all were able to put a nut on a bolt or brush their teeth, and some were able to button and unbutton where they couldn't do that before. One teenager who was previously completely aphasic spoke an entire sentence“ (Medscape)

- Neurology Live, “SB623’s Potential for Traumatic Brain Injury: Peter J. McAllister, MD, FAAN”
- Medscape, "Stem Cells Restore Lost Function in Traumatic Brain Injury"
- Practical Neurology, “Modified Stem-Cell Implants Improve Function in Chronic Traumatic Brain Injury”
- CGT Live, “Around the Helix: Cell and Gene Therapy Company Updates – April 6, 2022”
- The Pharma Letter, “SanBio’s SB623 demonstrated sustained improvement in motor impairment in brain injury”
- BioSpace, "AAN Spotlight: Multiple System Atrophy, Migraine, ALS and Parkinson’s"
- Trial Site News, “SanBio’s SSB623 Showed Sustained Improvement in Motor Impairment and Function in Traumatic Brain Injury Patients”
- BioSpace, “Argenx, Pharma Two B, SanBio Present at AAN”
- Seeking Alpha, “SanBio’s SB623 shows improvement in function in patients with brain injury in phase 2 trial”
- 日経バイオテク, 「ヘリオスがARDS承認申請の延期で急落、サンバイオの第2相データ解釈に治験医がコメント」
- 日刊工業新聞, 「再生細胞薬「SB623」、運動・日常動作が改善　サンバイオが米学会で解析結果」

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2. TBIフェーズ2試験最終解析結果

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EFFICACY AND SAFETY OUTCOMES IN PATIENTS WITH CHRONIC TRAUMATIC BRAIN INJURY: FINAL ANALYSIS OF THE PHASE 2 STEMTRA TRIAL

Peter McAllister MD, Benjamin M. Frishberg MD, Albert Lai MD, Takao Yasuhara MD, Steven C. Cramer MD, Masahito Kawabori MD, PhD, Michael C. Munin MD, Neil E. Schwartz, MD, PhD, Bijan Nejadnik, MD, Damien Bates, MD, PhD, Hideaki Imai MD, PhD, Alan H. Weintraub MD

STEMTRA: Study Design

- Phase 2, double-blind, randomized, surgical sham-controlled study of 1-year duration (NCT02416492)
- Stereotactic intracranial implantation of allogeneic modified bone marrow-derived mesenchymal stromal cells (SB623) in patients with stable chronic motor deficits secondary to TBI
- 61 patients at 13 surgical and 18 assessment sites in the US, Japan, and Ukraine
- Patients underwent stratified randomization in a 1:1:1:1 ratio to receive single doses of either $2.5x10^6$, $5x10^6$, $10x10^6$ SB623 cells or sham surgical procedure
- Physiotherapy: Subjects were instructed on of a set of exercises (cylinder grasp, thumb raise, stand and squat, walk) to be carried out at home every morning and afternoon for the first six months of the study, Screening (Visit 1) through Week 24 (Visit 8)
- **Primary efficacy endpoint was change from baseline in Fugl-Meyer Motor Scale (FMMS) score at 24 weeks among all patients who underwent surgery (N=61)**
- Safety follow-up 1-year

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STEMTRA: Patient Population

- Patients aged 18-75 years with chronic motor deficit secondary to stable TBI (≥12 months post-TBI)
  - GOS-E score of 3-6 (i.e., moderate or severe disability)
  - Require Motricity Index 10-81 (UE Scale) and/or 10-78 (LE Scale) with at least two scores <33 of which one score <25, and at least one score >0
- Focal cerebral injury identified on MRI (± diffuse axonal injury) correlated with clinical motor deficit
- Able to undergo all planned neurological assessments (i.e., severe cognitive impairment, orthopedic deficits, or pain may exclude)
- Able and willing to undergo CT and MRI
- No seizures in prior 3 months

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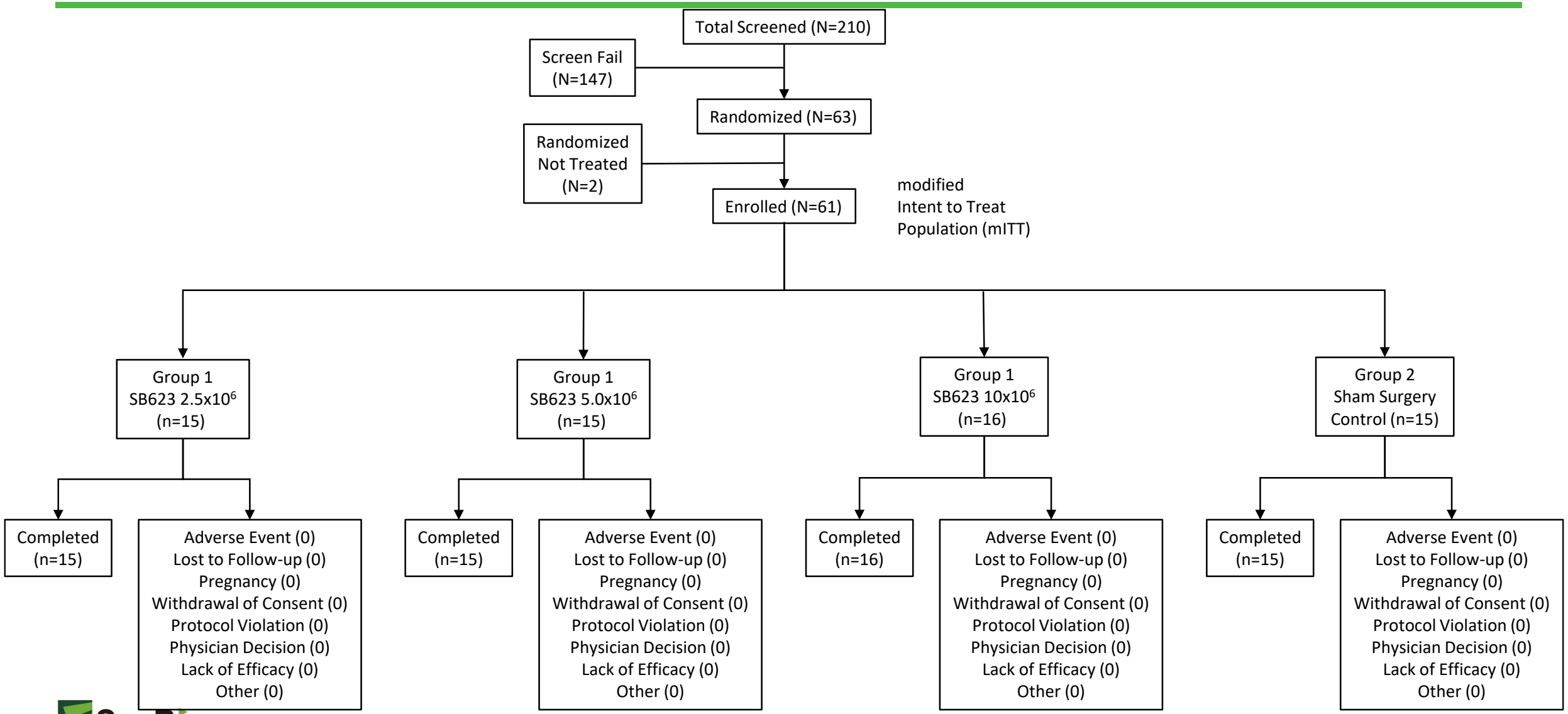
STEMTRA: Patient Disposition



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STEMTRA: Baseline Demographics

	SB623 Cell Dose/Implantation				Control (n=15)	Total (N=61)
	$2.5x10^6$ (n=15)	$5x10^6$ (n=15)	$10x10^6$ (n=16)	Pooled (n=46)		
Age (years)						
Mean (SD)	36.7 (13.6)	31.2 (9.2)	34.2 (11.5)	34.0 (11.5)	35.5 (13.0)	34.4 (11.8)
Median	34.0	30.3	30.2	32.6	35.4	33.4
Range: Min-Max	19.8-65.2	18.5-53.1	18.9-53.0	18.5-65.2	18.8-67.5	18.5-67.5
Gender, n (%)						
Male	11 (73.3)	12 (80.0)	11 (68.8)	34 (73.9)	9 (60.0)	43 (70.5)
Female	4 (26.7)	3 (20.0)	5 (31.3)	12 (26.1)	6 (40.0)	18 (29.5)
Time Since Injury (months)						
Mean (SD)	103.9 (68.0)	82.0 (67.9)	94.3 (76.4)	93.6 (10.6)	99.3 (23.1)	95.0 (9.7)
Median	86.5	42.6	69.7	72.9	62.4	68.9
Range: Min-Max	20.2-242.2	19.0-240.1	16.8-341.2	16.8-341.2	28.0-336.7	16.8-341.2
Race, n (%)						
White	11 (73.3)	9 (60.0)	11 (68.8)	31 (67.4)	11 (73.3)	42 (68.9)
Black	0 (0.0)	1 (6.7)	0 (0.0)	1 (2.2)	0 (0.0)	1 (1.6)
Asian	4 (26.7)	5 (33.3)	5 (31.3)	14 (30.4)	4 (26.7)	18 (29.5)
Ethnicity, n (%)						
Hispanic or Latino	0 (0.0)	1 (6.7)	1 (6.3)	2 (4.3)	0 (0.0)	2 (3.3)
Not Hispanic or Latino	15 (100.0)	14 (93.3)	15 (93.8)	44 (95.7)	15 (100.0)	59 (96.7)

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STEMTRA: Outcome Measures

- Impairment and functional limitation measures
  - Fugl-Meyer Motor Scale (FMMS): primary endpoint at 24 weeks
- Activity and activity limitation measures
  - Action Research Arm Test (ARAT)
  - Gait Velocity
  - NeuroQOL Upper and Lower Extremity Function T Scores
- Participation and participation restriction measures
  - N/A
- Measures that cross ICF domains
  - Disability Rating Scale (DRS)

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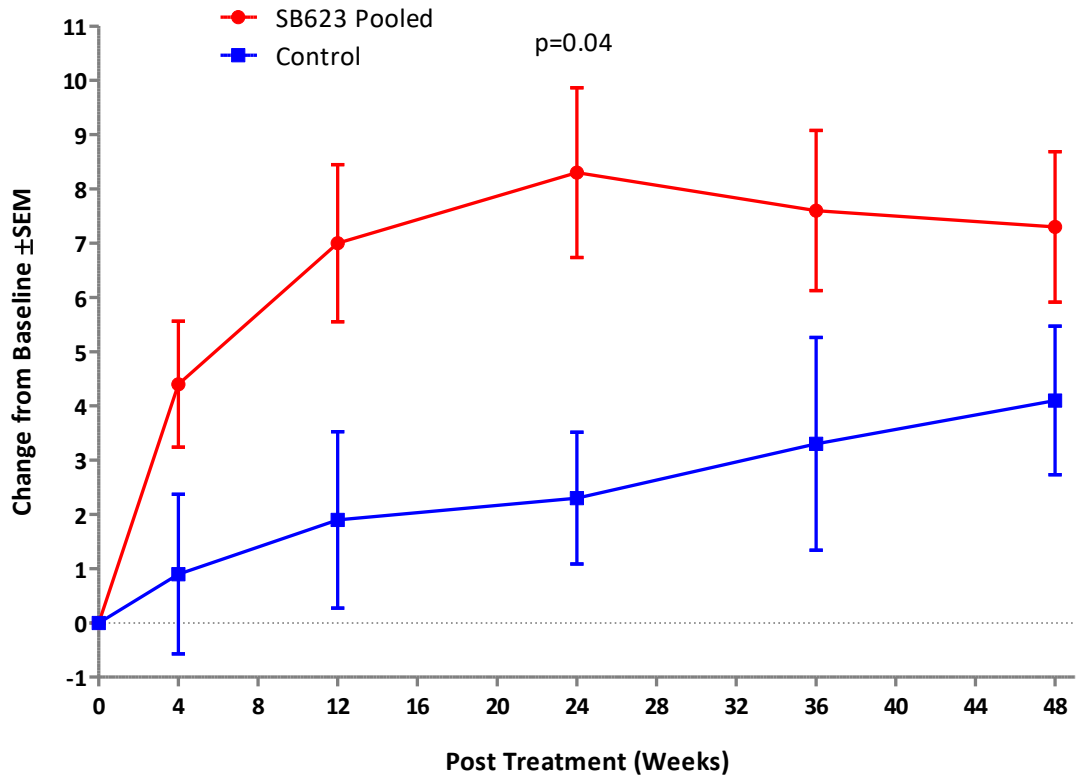
STEMTRA: Fugl-Meyer Motor Scale (FMMS)

- **Primary efficacy endpoint was achieved**
- Change of FMMS score from baseline was significantly higher for SB623-treated compared to control patients at 24 weeks
- Least square mean (SE) at 24 weeks:

  8.3 (1.4) vs. 2.3 (2.5), p=0.04
- Change of FMMS score from baseline was not significantly different for SB623-treated compared to control patients at 4, 12, 36, and 48 weeks
- Least square mean (SE) at 48 weeks:

  7.5 (1.3) vs. 4.1 (2.2), p=0.20
- Change of FMMS score from baseline was significant for SB623-treated but not control patients at 48 weeks



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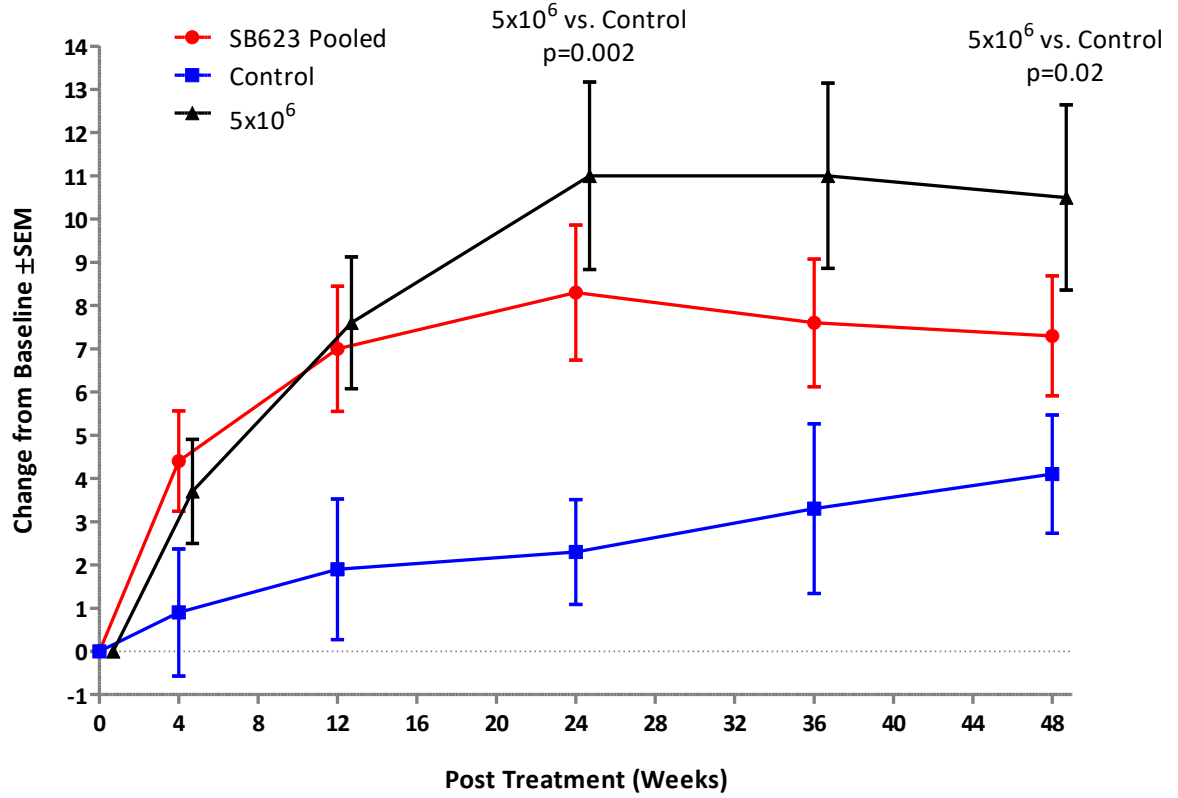
STEMTRA: FMMS 5x10^6 vs. Control at 24 and 48 Weeks

- Change of FMMS score from baseline was significantly higher for the 5x10^6 SB623 group (n=15) compared to control patients at 24 weeks (p=0.002) and 48 weeks (p=0.02)
- 5x10^6 SB623 dose will be the focus for future clinical development



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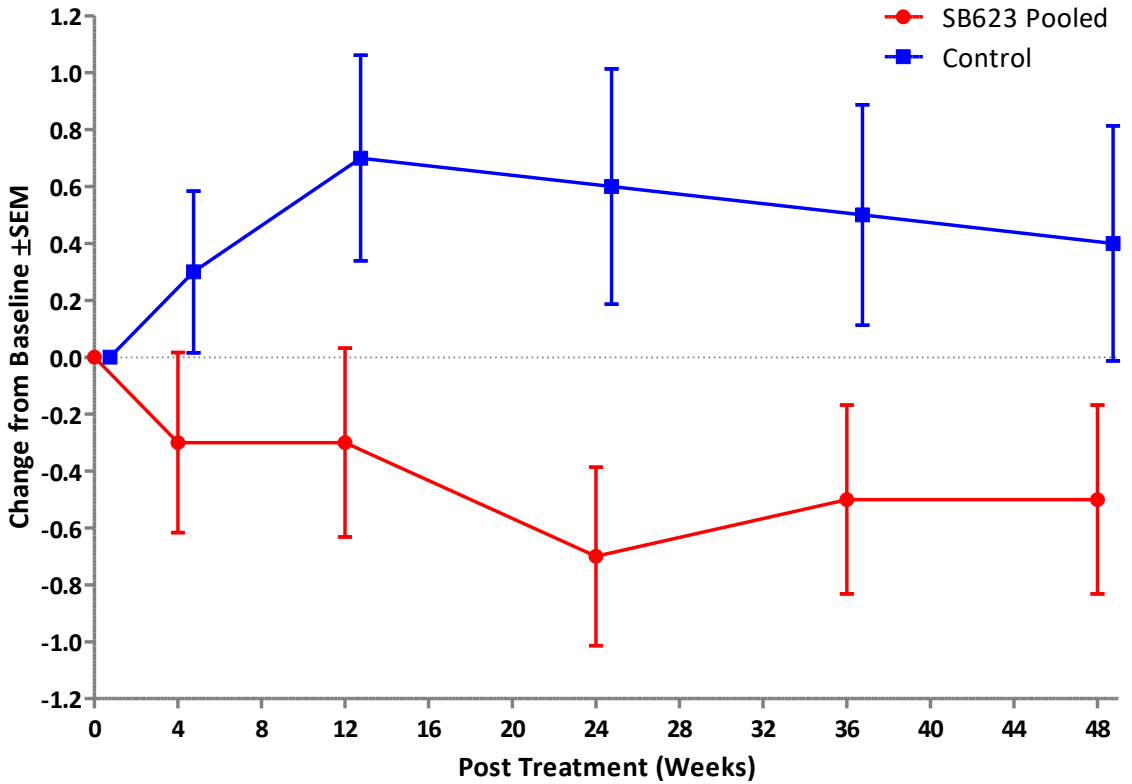
STEMTRA: Disability Rating Scale (DRS)

- Scores on DRS items include values from 0 to 29 (low to high level disability) and address impairment (eye opening, communication ability, motor response), disability (cognitive abilities for feeding, toileting, and grooming), and handicap (level of functioning and employability)
- Change of DRS score from baseline was greater for SB623-treated compared to control patients at Weeks 4 to 48
- Significant difference from baseline in the SB623 treatment arm at 24 weeks



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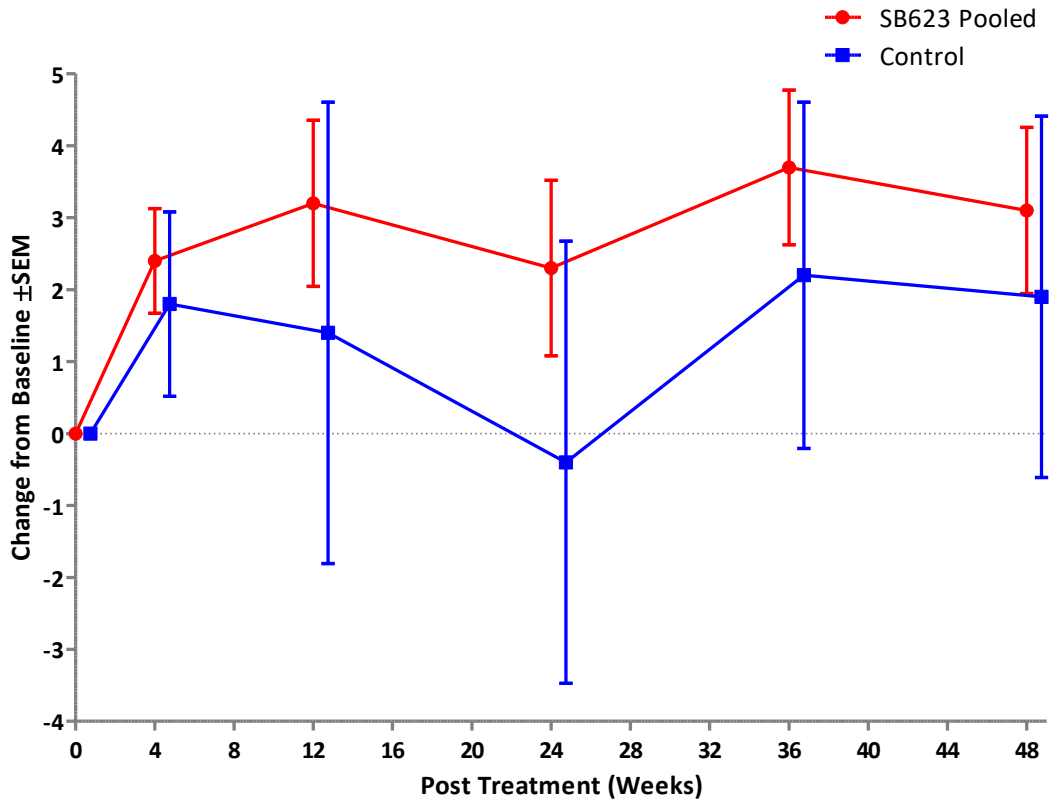
STEMTRA: Action Research Arm Test (ARAT)

- ARAT consists of 19 items organized in four subscales (grasp, grip, pinch, and gross movements) scored by a four-level ordinal scale ranging from 0 (no movement) to 3 (normal movement), maximum ARAT score is 57, corresponding to normal upper limb function
- Change of ARAT score from baseline was greater for SB623-treated compared to control patients at Weeks 4 to 48
- More SB623-treated than control patients achieved ARAT change of ≥6 points from baseline at 24 weeks: 19.5% vs. 14.3%
- Significant difference from baseline in the SB623 treatment arm at 48 weeks



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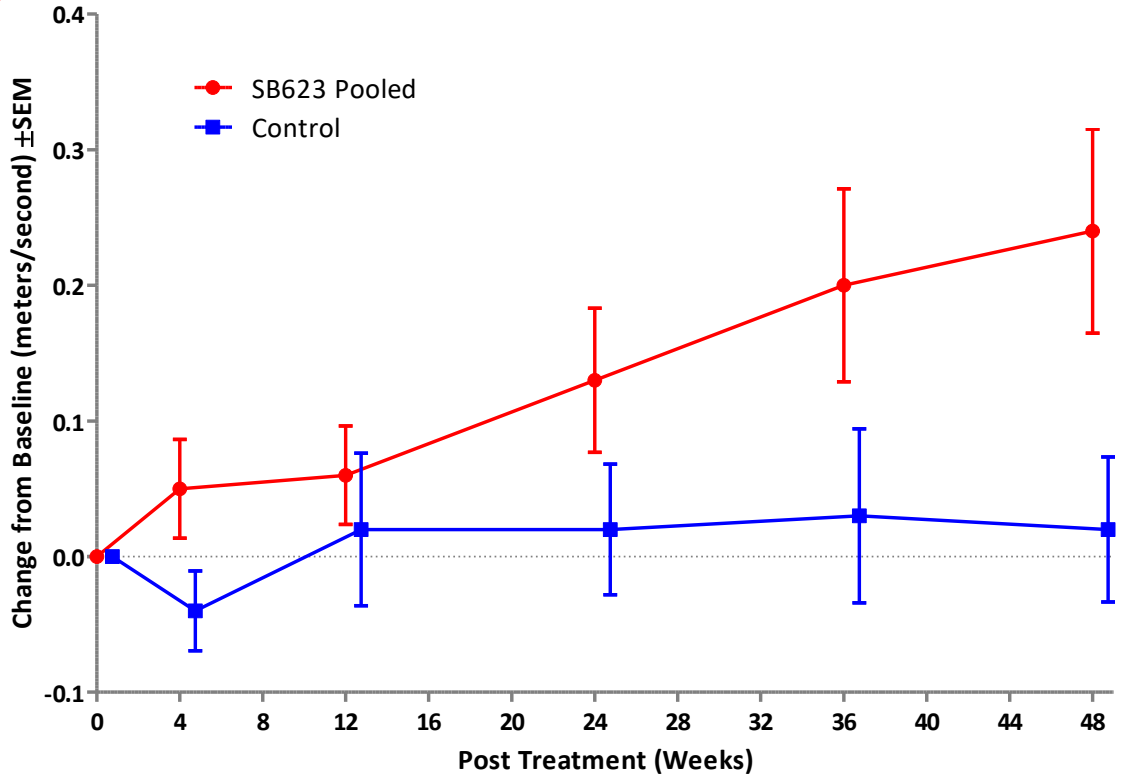
STEMTRA: Gait Velocity

- Gait velocity is used to assess gait speed over a distance of 10 meters
- Change of gate velocity from baseline was greater for SB623-treated compared to control patients at 4 to 48 weeks
- Significant difference from baseline in the SB623 treatment arm at 24 and 48 weeks



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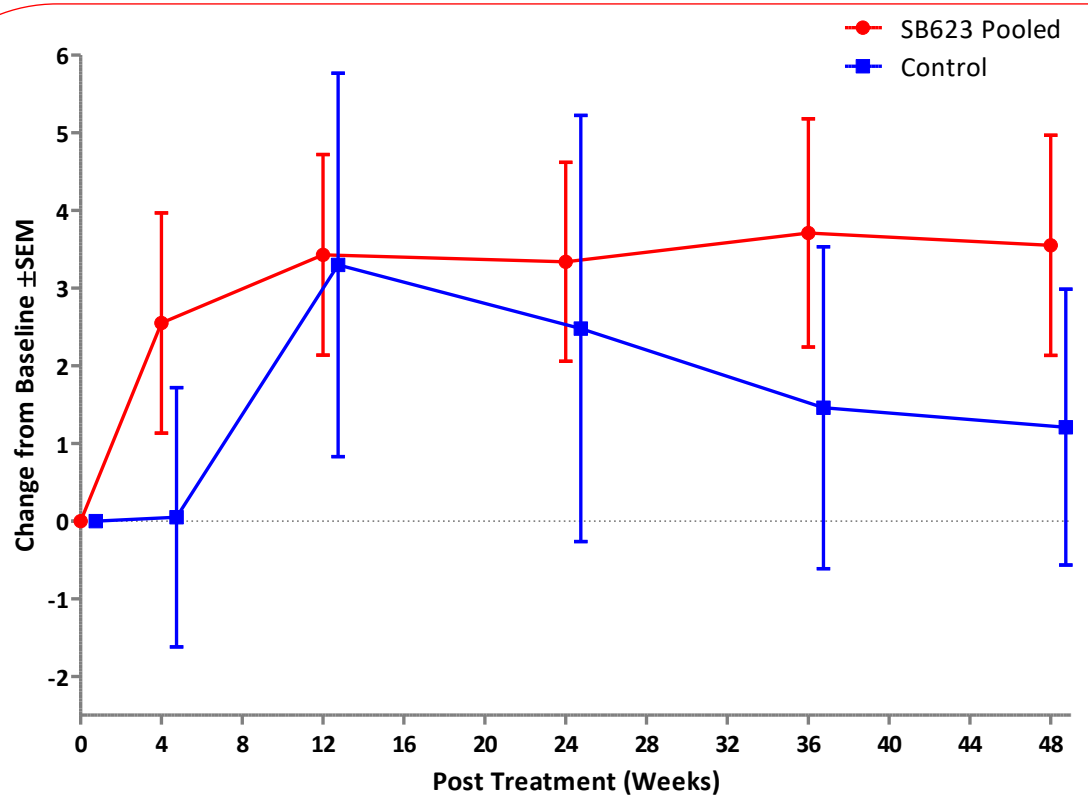
STEMTRA: NeuroQOL Upper Extremity Function T Score

- NeuroQOL is a set of self-report measures which assess HRQOL of adults with neurological disorders
- Upper extremity function subdomain (fine motor, activities of daily living [ADL]) evaluates ability to carry out various activities involving digital, manual, and reach-related functions, ranging from fine motor to self-care (ADL)
- Change of NeuroQOL upper extremity function T score from baseline was greater for SB623-treated compared to control patients at Weeks 4 to 48
- Significant difference from baseline in the SB623 treatment arm at 24 and 48 weeks



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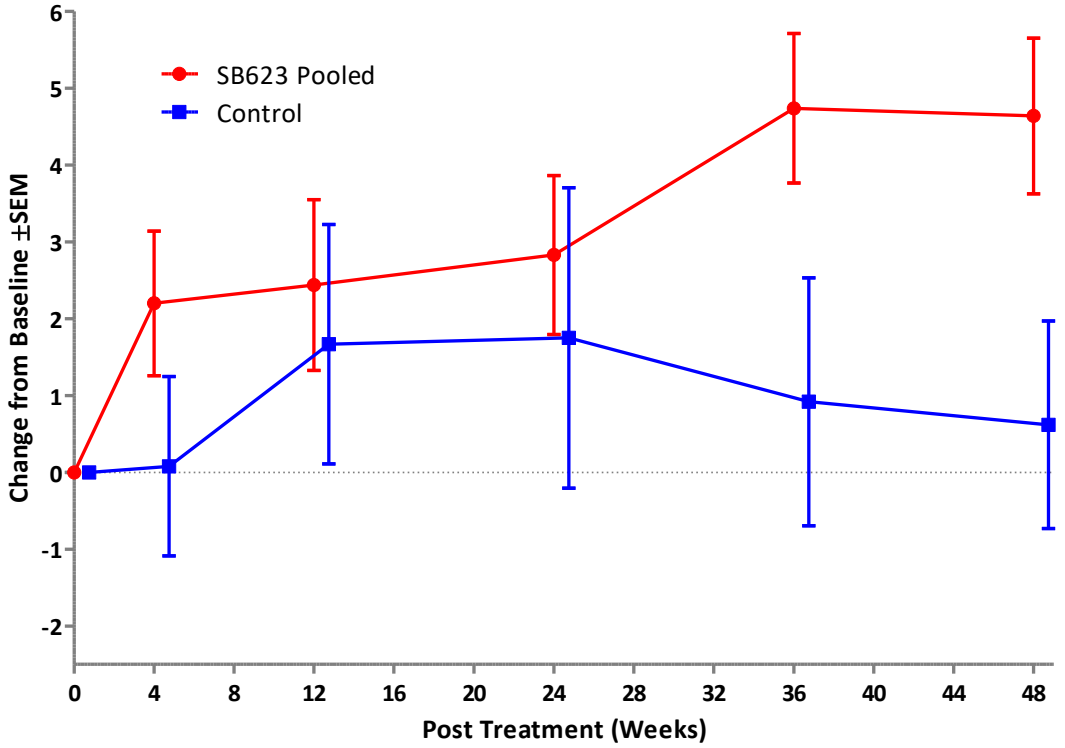
STEMTRA: NeuroQOL Lower Extremity Function T Score

- NeuroQOL is a set of self-report measures which assess HRQOL of adults with neurological disorders
- Lower extremity function subdomain evaluates ability to carry out various activities involving the trunk region and increasing degrees of bodily movement, ambulation, balance or endurance
- Change of NeuroQOL lower extremity function T score from baseline was greater for SB623-treated compared to control patients at Weeks 4 to 48
- Significant difference from baseline in the SB623 treatment arm at 24 and 48 weeks



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STEMTRA: Adverse Events (AEs)

- **At 48 weeks:**
  - All patients experienced at least one AE
  - There was no significant difference in the rate of AEs between pooled SB623-treated and control patients
  - There was no relationship between cell dose and frequency of AEs
  - No patients withdrew due to adverse events
  - There were no dose-limiting toxicities or deaths

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STEMTRA: Adverse Events

Treatment Group	Not Related (%)	Unlikely Related (%)	Possibly Related (%)	Probably Related (%)	Definitely Related (%)	Total Number of Events, n (%)
Relationship to Treatment						
SB623	74.8	19.1	5.3	0.8	0	246 (100.0)
Control	75.3	19.8	4.9	0	0	81 (100.0)
Relationship to Procedure						
SB623	54.9	6.9	12.6	13.0	12.6	246 (100.0)
Control	59.3	9.9	14.8	3.7	12.3	81 (100.0)

- Over 90% of AEs in both SB623 and control groups were assessed as being not related or unlikely to be related to treatment
- Over 30% of AEs in both SB623 and control groups were assessed as being possibly, probably, or definitely related to procedure

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STEMTRA: Serious Adverse Events

Cell Dose/ Implantation	Serious Adverse Event	Relationship to Treatment	Relationship to Procedure
$2.5x10^6$	Patient 1: Delirium (post-operative Days 3-7)	Not related	Not related
$5x10^6$	Patient 2: Transient ischemic attack (post-operative Days 97-106)	Not related	Not related
$10x10^6$	Patient 3: Seizure (post-operative Day 66-67)	Unlikely related	Possibly related
$10x10^6$	Patient 3: Seizure (post-operative Day 360-367)	Not related	Not related
$10x10^6$	Patient 4: Delirium (post-operative Days 1-3)	Possibly related	Probably related
$10x10^6$	Patient 4: Worsening of poor balance (post-operative Day 136 and ongoing)	Unlikely related	Probably related
Control	Patient 5: Wound infection (post-operative Days 153-170)	Not related	Definitely related
Control	Patient 6: Bicycle fall (accident) (post-operative Days 148-149)	Not related	Not related
Control	Patient 7: Seizure (post-operative Day 227)	Unlikely related	Unlikely related

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STEMTRA: Trial Summary

- 61 patients successfully underwent SB623 implantation or sham surgery and the primary efficacy endpoint was achieved:
  - Change of FMMS score from baseline at 24 weeks (primary efficacy endpoint) was significantly higher for SB623-treated patients compared to control patients (p=0.04)
  - FMMS, ARAT, Gait Velocity, and NeuroQOL upper and lower extremity function T scores were significantly improved from baseline in SB623-treated patients, and were greater than controls, however, differences were not statistically significant compared to control patients at 48 weeks
  - Change of FMMS score from baseline was significantly higher for the $5x10^6$ SB623 dose than control at 24 and 48 weeks, and will be the focus for future clinical development
  - The primary end point for the study was for 24 weeks, however, the study was extended to 48 weeks to collect more safety data
  - Despite the fact that the study was not powered for 48 weeks efficacy, the primary endpoint difference of the control group and the $5x10^6$ SB623 dose group (which has been selected for the phase 3 TBI) was statistically significant
  - SB623 cell implantation was associated with not only improvement of motor impairment but also improvement of function and activities of daily living at 48 weeks
- Implantation of SB623 cells was safe and well tolerated
  - No significant difference in AEs or SAEs between groups
  - No deaths or dose-limiting toxicities noted

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Thank You!

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3. 質疑応答

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