SpinalCure Australia

Tuesday 9th January, 2018

U.S. National Institutes of Health & Australian/New Zealand Clinical Trial Registry Database Records for Current Clinical Drug Trials

'Improving Functional Outcomes in Spinal Cord Injury'

Summary Report, Part 1

Phase 1 Studies:

1. **Neuralstem Inc** is continuing their **Phase 1** study of **human spinal cord-derived neural stem cell** (HSSC) transplantation for the treatment of chronic SCI. The study isrecruiting. Delivery of NSI-566 neural stem cells into the spinal cord will be accomplished using well-established stereotactic injection procedures. Completion date noted as March 2016. Last updated, September, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 1. NCT01772810

2. The **Shirley Ryan AbilityLab (Rehabilitation Institute of Chicago)** is conducting a **Phase 1** study on **monoaminergic modulation** of motor function in subacute incomplete SCI. They hypothesise that administering the SSRI antidepressant drug, Lexapro, early following SCI may facilitate independent stepping ability and improve locomotor recovery if the drug treatment is combined with intensive stepping training. Treatment with Lexapro may help to strengthen remaining nerve connections along the spine, giving patients more muscle control during rehabilitation exercises. The recruitment status is **active, not recruiting**. Last updated June 14 2016. See detail regarding the clinical trial in Summary Report Part 2, page 2. NCT01753882

3. The **Shirley Ryan AbilityLab (Rehabilitation Institute of Chicago)** is continuing their **Phase 1** study on **serotonergic modulation** of motor function in subacute and chronic SCI (Recruitment status: Active, not recruiting). The investigators are studying the effects of Lexapro on strength and walking ability following human motor incomplete SCI. Using detailed electrophysiological recordings, in addition to measuring biomechanics and behaviour, the investigators are testing the effects of Lexapro on motor behaviours during static and dynamic conditions. Last updated December, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 3. NCT01788969

4. **Charite University**, Berlin, has completed a **Phase 1** safety study of using ibuprofen to treat acute SCI. The trial seeks to investigate the tolerability and feasibility of "small molecule" **(ibuprofen) mediated Rho-inhibition** as a putative neuroprotective, plasticity-enhancing and neurorestorative intervention. Last updated October, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 4. NCT02096913

5. **CSL Behring AG and The University of Queensland** have registered a **Phase 1** trial assessing the feasibility, safety, and efficacy of IVIg therapy in patients with acute traumatic spinal cord injury. Registered in October 2016, the trial is not yet recruiting. See detail regarding the clinical trial in Summary Report Part 2, page 5. ACTRN1261600138543

6. **Tri Phuoc Biotechnology** (Vietnam) was recruiting participants for a **Phase 1 | Phase 2** randomised control trial designed to assess the safety and effect of autologous **adipose derived stem cell (ADSCs)** transplantation in acute SCI patients. Current status **unknown** as information has not been verified recently. Part of their assessment will include determining whether functional outcome is improved following ADSCs transplantation in acute SCI patients, using pre-transplantation spinal cord function as the control. Selection of patients began in February 2013, and the study was due for completion March 2015. Last updated January, 2014. See detail regarding the clinical trial in Summary Report Part 2, page 7. NCT02034669

7. **StemCells, Inc** conducted a **Phase 1 | Phase 2** study of the safety and preliminary efficacy of intramedullary spinal cord transplantation of **human neural stem cells** (**HuCNS-SC®**) in subjects with thoracic (T2-T11) spinal cord trauma. The trial was conducted in Zurich, Switzerland and is now complete (as of April 2015). The clinical protocol also included patients with the less severe, incomplete injuries. No study results posted, last updated June 2015. See detail regarding the clinical trial in Summary Report Part 2, page 8. NCT01321333

8. **Kringle Pharma, Inc** (Japan) is recruiting participants for a randomised, double-blind, placebo-controlled **Phase 1 | Phase 2** study designed to evaluate safety and efficacy of **KP-100IT**, code of Hepatocyte Growth Factor (HGF) formulation for intrathecal injection, as a treatment for acute SCI. The study is conducted at two clinical sites in Japan. Last updated October, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 9. NCT02193334

9. **Translational Biosciences** planned to conduct a safety and feasibility trial **Phase 1 | Phase 2** using **human umbilical cord-derived mesenchymal stem cells** (UC-MSC) and bone marrow mononuclear cells (BMMC) from patients with SCI and injecting them back into the spinal fluid intrathecally and intravenously. The trial was withdrawn prior to enrollment. Last updated August 2017. See detail regarding the clinical trial in Summary Report Part 2, page 10. NCT02237547

10. The **University of California Los Angeles** is currently recruiting for a **Phase 1 | Phase 2** trial to assess the efficacy of spinal cord neuromodulation to improve movement in patients with SCI. The study aims to evaluate whether **epidural stimulation** and an agonist drug can improve motor function, as assessed by formal arm/hand motor testing. Last updated January 2016. See detail regarding the clinical trial in Summary Report Part 2, page 11. NCT02313194

11. **Asterias Biotherapeutics** is recruiting for a **Phase 1 | Phase 2** trial of **AST-OPC1** in patients with cervical complete SCI. The purpose of the study is to evaluate the safety of 3 sequential escalating doses of AST-OPC1 and assess neurological function in these patients. Last updated December 2017. See detail regarding the clinical trial in Summary Report Part 2, page 12. NCT02302157

12. **Ferrer Internacional S.A.** are recruiting participants for a **Phase 1 | Phase 2** randomized, double-blind, interventional study to the evaluate the safety and tolerability of FAB117-HC - a medicinal product containing human allogeneic adipose derived adult mesenchymal stem cells expanded and pulsed with H2O2 (HC016 cells) - administered at a single-time point to patients with acute thoracic traumatic **spinal cord injury.** The study will also include initial exploration of potential clinical efficacy. Dose levels of 20 million and 40 million cells will be administered. Last updated

February 2017. See detail regarding the clinical trial in Summary Report Part 2, page 13. NCT02917291

13. **Banc de Sang I Teixits** are conducting a **Phase 1 | Phase 2** trial in which the efficacy of data in intrathecal administration of expanded **Wharton's jelly mesenchymal stem cells** is investigated, in patients with chronic traumatic spinal cord injury. This trial was received December 14, 2016 and last updated in May 2017. See detail regarding the clinical trial in Summary Report Part 2, page 14. NCT03003364

14. **T**he **University of California Los Angeles** is currently recruiting participants for an early **Phase 1** non-randomised trial to improve bladder function in patients with SCI by neuromodulation. The trial will investigate the safety and utility of spinal cord neuromodulation to improve bladder function in the context of spinal cord injury. Last updated January 26, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 15. NCT02331979

15. **Ohio State** is currently recruiting for their **Phase 1 | Phase 2** trial addressing the safety of intravenous treatment of acute traumatic cervical spinal cord injury (SCI) with **RP-1127**. Outcomes measured are the number of patients experiencing adverse events after administration. Last updated March 23, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 16. NCT02524379

Phase 2 Studies:

16. **Karolinska University Hospital** is conducting a **Phase 2**, single center, open-label, non-randomized clinical study to assess the uptake, safety and tolerability of **Imatinib** in acute cervical SCI patients. Not yet open for participant recruitment, expected enrollment, 10. Last updated February, 2015. See detail regarding the clinical trial in Summary Report Part 2, page 17. NCT02363361

17. **Daiichi Sankyo Inc** has completed the multicentre, randomised, double-blind, placebo-controlled, parallel-group **Phase 2** trial. The study evaluated the efficacy, safety, and pharmacokinetics of the **SUN13837** injection in adult subjects with acute SCI. No study results have been posted; last updated August 23, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 18. NCT01502631

18. The **University of Florida** is collaborating with the Department of Veterans Affairs in a **Phase 2** trial to assess whether **testosterone plus finasteride** treatment will improve neuromuscular function in men who have experienced ambulatory dysfunction subsequent to incomplete SCI This study is currently recruiting participants. Last updated December 7, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 19. NCT02248701

19. **Neurogen Brain and Spine Institute** is recruiting for a **Phase 2** study on the effect of stem cell therapy on common symptoms in patients with spinal cord injury. This record was updated in September, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 21. NCT02009124.

20. **The University of British Columbia** is recruiting for a **Phase 2** trial addressing Autonomic Dysreflexia (AD), drug intervention: **Fesoterodine**. Current enrolment of 20, measuring self-reported function and frequency of AD among other outcomes. Last updated November 1, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 22. NCT02676154

21. **AOSpine North America Research Network** is recruiting for a multi-centre, randomised, placebo controlled, double-blinded, **Phase 2 | Phase 3** trial of efficacy and safety of **Riluzole** in acute SCI. The aim of this study is to evaluate efficacy and safety of riluzole in improving neurological motor outcomes of patients with acute SCI at 6-months post injury. Last updated June 5, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 23. NCT01597518

22. **Vertex Pharmaceuticals Incorporated** are recruiting for a **Phase 2 | Phase 3** double-blind randomised placebo controlled study to assess the efficacy and safety of VX-210 in subjects with **acute traumatic spinal cord injury**. Primary outcome measures change from baseline in upper extremity motor score (UEMS) six-months post treatment. This study is due to be completed June 2018. Last updated October 30, 2017. See detail on trial at page 24. NCT02669849

23. **Da Nang Hospital** are recruiting for a **Phase 2** study investigating the safety profile and adverse effects of the using **bone marrow derived mononuclear cells** for spinal cord injury. This trial was first received October 3, 2016 and was last updated January 9, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 25. NCT02923817

Phase 3 Studies:

24. The **University of Calgary** are currently recruiting patients for inclusion in a **Phase 3** double-blind randomised clinical trial in acute SCI patients, investigating whether maintenance of normotension (MAP ≥ 65mmHg) is inferior to **induced hypertension** (MAP ≥ 85mmHg) for 7 days following **acute** SCI. Last updated October, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 26. NCT02232165

25. **Hospital Nacional de Parapléjicos de Toledo** (Spain) are conducting a triple-blinded, randomised, placebo-controlled **Phase 3** trial to evaluate the efficacy and safety of one year of treatment based on daily doses of exogenous **growth hormone** (GH) in patients with traumatic SCI. The study status is unknown. Last updated January 9, 2015. See detail regarding the clinical trial in Summary Report Part 2, page 27. NCT01329757

26. The **Rick Hansen Institute Canada Inc** is conducting a **Phase 3** clinical trial to assess the efficacy of IV **minocycline** in improving neurological and functional outcome after acute non-penetrating traumatic spinal cord injury (SCI). The University of Calgary and Alberta Paraplegic foundation are listed as collaborators. The primary hypothesis is that motor recovery will be improved through intravenous administration of minocycline twice daily administered to subjects with acute traumatic non-penetrating cervical SCI starting within 12 hours of injury. Last updated October, 2014. See detail regarding the clinical trial in Summary Report Part 2, page 28. NCT01828203

27. **Baylor College of Medicine** is currently recruiting for a **Phase 3** trial to assess the safety and efficacy of **intra-detrusor injected onabotulinumtoxin A** versus oral oxybutynin in SCI patients with neurogenic detrusor overactivity. The purpose of the trial is to determine whether injected onabotulinumtoxin A is effective for the treatment of urinary incontinence, and it performance compared to a drug taken orally. Last updated August 29, 2016. See detail regarding the clinical trial in Summary Report Part 2, page 30. NCT01050114

28. **Ipsen** are recruiting participants for **Phase 3** trial delivering drug intervention and observing any change in weekly urinary incontinence episodes. The aim being to provide confirmatory evidence of the safety of **two Dysport®** doses, compared to a placebo for the treatment of urinary incontinence. This study was last updated December, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 31. NCT02660359

29. **InVivo Therapeutics** is conducting a **Phase 3** trial for **neuro-spinal scaffold** in acute thoracic SCI. This study is active, but not recruiting. Their purpose is: *To evaluate whether the Scaffold is safe and feasible for the treatment of complete functional spinal cord injury as determined by no degradation in paralysis level or sensory motor neurological function beyond that typically seen in the AIS.* Last updated August 2017. See detail regarding the clinical trial in Summary Report Part 2, page 32. NCT02138110

Phase 4 Studies:

30. The **Rick Hansen Institute Canada Inc** is also conducting a **Phase 4** clinical trial to assess the efficacy of intradetrusor-injected Botox in ameliorating episodes of autonomic dysreflexia in individuals with chronic SCI. The recruitment status of this study is unknown, as the information has not been verified lately. Last updated November 19, 2014. See detail regarding the clinical trial in Summary Report Part 2, page 33. NCT02298660

31. **Moleac Pte Ltd.** is recruiting for a **Phase 4** study investigating the tolerability, safety and efficacy of **NeuroAid** in patients with Spinal Cord Injury. The study aims to measure Spinal Cord Injury severity, motor recovery and adverse events. Last updated August 28, 2015. See detail regarding the clinical trial in Summary Report Part 2, page 34. NCT02537899

32. **Oslo University Hospital** and **Sunnaas Rehabilitation Hospital** are conducting a **Phase 4** trial to assess whether **onabotulinumtoxin A** can be used to prevent bladder dysfunction in acute SCI. The trial is currently recruiting, and seeks to explore the effect of early treatment with onabotulinumtoxin A in patients with acute complete SCI on the development of neurogenic detrusor over-activity. Last updated February, 2017. See detail regarding the clinical trial in Summary Report Part 2, page 35. NCT01698138