



Australasian Faculty of  
Rehabilitation Medicine



The Royal Australasian  
College of Physicians

## **AUSTRALASIAN FACULTY OF REHABILITATION MEDICINE (AFRM) POSITION STATEMENT ON THE USE OF STEM CELLS AS A THERAPY FOR CEREBRAL PALSY**

### **Cerebral Palsy**

Cerebral palsy (CP) is an umbrella term describing a group of conditions that together constitute the most common cause of physical disability in childhood, affecting approximately 2 per 1000 live births in Australia.

CP is defined as:

- A permanent disorder of the development of movement and posture causing activity limitation;
- It is attributed to a non-progressive disturbance that occurred in the developing foetal or infant brain;
- The motor disorders are often accompanied by disturbances of sensation, perception, cognition, communication and behaviour; by epilepsy and by secondary musculoskeletal problems<sup>[1]</sup>.

CP may be caused by many factors including disorders of early brain development, and infectious, inflammatory, traumatic or vascular insults that may occur at any time during pregnancy or in infancy<sup>[2]</sup>. There is no known cure for CP and 17 million worldwide are affected by this condition.

Clinics in several countries currently offer stem cell therapies to children with CP, and some Australian families are utilizing the services of these clinics. There is concern that treatments offered in these clinics may not be based on experiments conducted with scientific rigour, and that sufficient safety controls may not be in place<sup>[3]</sup>. This has prompted the need for a Position Paper from the medical community on the topic.

### **What are stem cells?**

Stem cells are precursor cells that can divide to produce either more identical stem cells, or many different cell types in the body<sup>[3]</sup> for example blood cells, muscle cells or nerve cells<sup>[4]</sup>. These cells can be either for growth and development, or for replenishment and repair. Stem cells occur at all stages of human development, from embryo to adult, but their versatility and numbers decrease with age.

Stem cells offer promise and the potential to treat a number of conditions. However, at present, the only scientifically proven stem cell treatment is haemopoietic stem cell transplantation<sup>[3]</sup>. This form of treatment began in the late 1950s and is a standard treatment for disorders of the blood and immune system such as leukaemia and lymphoma.

## Why stem cell therapy for CP

Stem cells have provoked considerable interest as a potential therapy for CP due to their potential to replace or support damaged brain tissue. Originally it was thought that stem cells might differentiate into either nerve cells or support cells, but this does not appear to happen in the human body. If stem cells are given at the right time, it is possible that they may prevent some of the damage to the brain by modifying the inflammatory response<sup>[5-7]</sup> and protecting nerve cells from death<sup>[8, 9]</sup>. Some types of stem cells may also help the brain to improve blood flow by forming new blood vessels<sup>[8, 10, 11]</sup>.

## What current research is available for families and health professionals?

A recent uncontrolled pilot study conducted in Seoul, Korea involving 20 children suggested the safety and feasibility of autologous cord blood infusion as a potential treatment for CP. This study reported some neurological improvements<sup>[12]</sup>.

There has been great interest in another more recently published Korean trial, one that was double-blinded, randomised with placebo control, using allogeneic umbilical cord blood cells in combination with erythropoietin. The study had three arms: (i) umbilical cord blood cells, erythropoietin, and rehabilitation; (ii) erythropoietin and rehabilitation; (iii) rehabilitation only. Immunosuppression was used in order to allow for the use of allogeneic cells. Compared with the erythropoietin and control groups, the group that received umbilical cord blood cells demonstrated statistically significant improvements in motor and cognitive performance. The incidence of serious adverse events did not differ between the groups. However, it should be noted that no group had umbilical cord blood cells alone, the reported follow up period was only six months and the cyclosporine used for immunosuppression may have had some impact on the results<sup>[13]</sup>.

There are two ongoing trials in the United States further examining the safety and efficacy of autologous umbilical cord blood for CP (Duke University<sup>[14]</sup> and Georgia Regents University<sup>[15]</sup>).

In addition, two comparative trials were announced recently. The first comparing autologous cord blood cells, autologous bone marrow cells, or placebo, to be conducted at University of Texas Health Science Center, USA<sup>[16]</sup>; the other comparing intrathecal infusion of mesenchymal stem cells derived from allogeneic cord blood with either one year of rehabilitation therapy or with normal clinical care, to be conducted at the General Hospital of Chinese Armed Police Forces<sup>[17]</sup>.

## Implications for families

If families are considering stem cell therapy overseas, they should take into account the lack of proven efficacy as well as the safety and costs associated with the procedure. This is especially important since there are concerns that centres offering these treatments may operate without adequate scientific rigour or safety controls in place. Clinics may use cells which are not well characterised and the risks associated with delivery methods may not be well explained. Undergoing such treatments may also disqualify patients from participating in future registered clinical trials.

It should be noted that regardless of whether stem cells are used in unregulated centres or as part of a recognised registered clinical trial, adverse reactions are possible including infection, trauma related to injection method and a theoretical risk of tumour development.

An Australian collaboration has formed to trial injecting autologous cord blood, but a feasibility study recently completed has demonstrated that the numbers of children with CP and stored cord blood are small<sup>[18]</sup>. Regardless, this collaboration is hoping to undertake

pilot studies with autologous umbilical cord blood and the use of matched sibling blood is also being considered.

## **Recommendations**

1. In view of the paucity of clinical trial outcomes, it is too soon to be able to make a decision about the possible usefulness of stem cell therapy for CP.
2. However, this is a rapidly developing field with more information regarding potential risks and benefits, likely to become available over the next few years.
3. It is recommended families discuss the matter with their physician.

To assist the patient and family/carer(s) make a well informed decision regarding the use of stem cell treatment for CP, more information is available on the listed websites below:

- [www.stemcellsaustralia.edu.au](http://www.stemcellsaustralia.edu.au) [last accessed on 23/05/2014]
- <http://www.stemcellfoundation.net.au/patient-information/handbook> [last accessed on 23/05/2014]
- [www.closerlookatstemcells.org/](http://www.closerlookatstemcells.org/) [last accessed on 23/05/2014]
- <http://anzctr.org.au/> [last accessed on 23/05/2014]
- <http://clinicaltrials.gov/> [last accessed on 23/05/2014]

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