FACT SHEET FOR CLINICIANS

UMBILICAL CORD BLOOD AS A TREATMENT FOR CEREBRAL PALSY

Cerebral Palsy ALLIANCE

Umbilical cord blood is emerging as a promising treatment option for children with cerebral palsy (CP). This fact sheet has been created for clinicians and healthcare professionals to explain why and how cord blood treatment is being used for CP, review the research evidence, detail the status of access to cord blood treatment and explore the future possibilities in Australia.

Current access to cord blood for CP

In 2024, the Therapeutic Goods Administration (TGA) approved access to autologous cord blood treatment for a child with CP via the Special Access Scheme (SAS) Category B pathway. This is the first known treatment outside of a clinical trial in Australia. The infusion took place in April 2025 at Monash Children's Hospital in Melbourne. SAS-Cat B approval was based on substantial evidence of the safety and efficacy of cord blood as a treatment for CP and similar compassionate access programs available overseas. CPA are now working with treating clinicians to understand and develop a process for additional families to access cord blood treatment via SAS going forward.

Outside Australia, Duke University in the US has a Food and Drug Administration (FDA)-authorised Expanded Access Program (NCT03327467), which has been active since 2017 and accepts international participants. Notably, in 2024, Duke started offering access to unrelated donor cord blood in addition to autologous and sibling units, substantially expanding eligibility.

Currently, there are no active clinical trials of cord blood treatment for CP in Australia, and limited international trials are recruiting. Additionally, private clinics offer access to cord blood and other cell therapies via medical tourism, often in countries with less rigorous regulatory environments.

Cerebral Palsy Alliance seeks to support access to evidence-based, safe and efficacious treatments for CP via regulated pathways, including clinical trials and SAS. Cord blood is one such new treatment option.

Cord blood for CP: the why and how

Several cell therapies, including umbilical cord blood cells, bone marrow cells, mesenchymal stromal cells, and neural stem cells, are currently under investigation as treatments for CP (Paton et al., 2021). Of these, cord blood is the most thoroughly studied, having been tested in clinical trials for 20 years. Cord blood, collected from the placenta, contains a variety of cell types in the mononuclear layer (or "buffy coat"), including haemopoietic stem and progenitor cells, mesenchymal stromal cells, endothelial progenitor cells, monocyte-derived and T-regulatory cells (Sun & Kurtzberg, 2021).

Preclinical evidence demonstrates that cord blood treatment promotes brain repair by reducing brain infarct size, astrogliosis, microglial activation, and neuroinflammation, and increasing the number of neurons and oligodendrocytes (Nguyen et al., 2023). These mechanisms are consistent with clinical findings after intravenous administration, whereby cord blood cells do not need to travel directly to the brain and instead work primarily via paracrine signaling to improve brain connectivity and functional motor outcomes (Novak et al., 2020; Sun et al., 2017). Notably, the application of cord blood for CP is distinctly different to historical use in haematology/oncology; for the treatment of blood cancers or blood conditions, the haematopoietic (blood forming) stem cells in cord blood engraft and replace bone marrow cells. In the case of cord blood infusions for CP, cord blood cells have a short half-life in peripheral circulation, do not engraft and treatment still results in sustained improvements in motor skills (Crompton et al., 2022; Paton et al., 2022).

Clinical evidence

20 year history in context

To date, 15 published clinical studies of cord blood treatment for CP have been conducted over 20 years. These include seven randomised controlled trials, involving 656 individuals with CP, 410 of whom received cord blood.

The available clinical trial data was recently synthesised in an Individual Participant Data Meta-Analysis (IPDMA), which aimed to assess the safety and efficacy of cord blood for improving gross motor function in children with CP, including exploring cell dose effect and responder subgroups (Finch-Edmondson et al, 2025).

Our Commitment to Data Collection

Institutes outside of Australia, e.g. Duke University, have a long history of cord blood infusions for CP. Whilst compassionate access programs collect and report on treatment safety, they are often under-resourced to measure outcomes that evaluate any potential treatment benefits.

CPA is committed to establishing an Australian treatment registry – to track outcomes, build an evidence base for public funding, and understand who benefits most.

Safety

Cord blood treatment for CP has a robust safety profile. Our recent IPDMA of 596 children with CP included in cord blood trials found comparable rates of serious adverse events between cord blood-treated (16%) and controls (13%), however, only one serious adverse event was found to be related to the cord blood treatment, specifically a reaction to the cryopreservative added to the cord blood unit (Finch-Edmondson et al, 2025). Consistent with the delivery of cryopreserved products, mild infusions reactions are seen to occur in 5-10% of cases. Importantly, these were treatable in all cases. Most children in this analysis (84%) received donor cord blood, rather than their own, indicating risk is not elevated by using donor cord blood. This is further supported by a systematic review of the use of donor cord blood across various neurological conditions (n=442 cord blood infusions) in which there were no longer-term safety concerns including reports of Graft-versus-host disease, or teratoma development (Paton et al., 2022). Consistent with standard protocols for administering blood products, the emphasis remains on ensuring an adequate environment for responding to any infusion reaction, monitoring for emerging infections and detecting product contamination.

Efficacy

The IPDMA demonstrated an overall, clinically meaningful effect of a single dose of intravenous cord blood for improving gross motor function in children with CP, 6-12 months post-treatment. Infusion of higher cell doses (of at least 55 million total nucleated cells per kilogram of bodyweight) was associated with increased effect size. Responder analyses identified that younger participants (5 years and under) with milder (ambulant, Gross Motor Function Classification System levels I-III) CP showed increased benefits for improving gross motor function. Studies investigating cord blood for CP have reported a range of outcomes across many domains (Finch-Edmondson et al., 2022).

Outside of motor function, there is only some evidence to date to suggest that cord blood may improve cognition in children with CP. Low-level evidence suggests that cord blood may be efficacious for improving other skills in older children and adults, or in those with more severe CP. Based on preclinical research, it is hypothesised that repeated cord blood treatments may provide added benefit. However, this has not been tested in clinical trials.

The treatment

The cord blood unit

Both allogeneic and autologous cord blood has been used for the treatment of CP, with comparable safety andefficacy. For allogeneic cord blood, the currently accepted standard is that the sibling must be at least a half (haploidentical) Human Leukocyte Antigen (HLA) match, and for an unrelated donor, ≥4/6 match. Cord blood infusion for CP should be administered in a hospital or clinical setting under the supervision of a medical team. Historically, cord blood treatment for CP has been administered in an outpatient day clinic, supported by transplant physicians.

The infusion

Prior to infusion, participant eligibility is confirmed to ensure they are medically stable, without infection and are a candidate for treatment. Importantly, a qualifying cord blood unit from either a public or private cord blood bank must be identified and selected. On the day of infusion, cord blood is thawed and prepared in ~50ml or less total volume. Cord blood is given via a peripheral intravenous line, without immune ablation. Immediately prior to infusion, medications such as low-dose anti-histamines (e.g., Benadryl) and steroids (e.g., Solu-medrol) may be given to help reduce any potential side effects caused by the cryopreservative in a cord blood unit (dimethyl sulfoxide, DMSO). The infusion itself takes approximately 20 minutes, followed by monitoring for any side effects with administration of fluids for approximately one hour.

Post-care and rehabilitation

All previous clinical trials have administered cord blood treatment in conjunction with standard care i.e., rehabilitation/physical therapy. As cord blood is hypothesised to provide benefit by reducing inflammation and optimising neuroplasticity, cord blood may help to provide a boost to the effects of rehabilitation for improving gross motor skills, and other outcomes. As such, it is recommended that cord blood treatment is accompanied by an evidence-based motor training intervention to maximise outcomes. Best practice guidelines indicate this is a dose of 14-40 hours of task-specific training (depending on the task to be trained) within a 6-week period. This dose can be achieved via a combination of face-to-face, virtual and/or home programs, with each mode shown to be equally effective. However, the intensity of therapy should be adjusted to accommodate the child's age (e.g., awake hours for infants). Task-specific training may be provided by a hospital, a private therapist, or NDIS-funded service provider.

Who is involved?

Neurologist / Paediatrician

In the case of SAS access to cord blood treatment, the child's treating specialist would confirm the CP diagnosis and whether the person is suitable for treatment, including confirming unit eligibility with the cord blood bank, and complete the SAS paperwork. The specialist would then liaise with haematology to coordinate the infusion and order any relevant tests or assessments.

Haematology and transplant physicians

Cord blood for CP should be administered under the supervision of a haematologist, transplant specialist or provider team. This is because they are trained in handling and giving blood or cell products and have existing processes to access/receive cord blood units ready for infusion. Moreover, as there is a risk of a reaction occurring during the treatment, they will be best placed to provide an appropriate and immediate medical response.

Physiotherapist / Occupational therapist

It is advised that cord blood infusion is accompanied by task-specific training to maximise potential benefits following cord blood infusion. This compliments the patient's regular rehabilitation schedule and may be provided by a hospital, a private therapist, or NDIS-funded service provider. In addition, some physical therapy teams may conduct baseline and follow up assessments focused on capturing motor outcomes.

Cord blood bank

Prior to treatment, a suitable umbilical cord blood unit must be obtained from the cord blood bank. Which unit to use might depend on the number of cells in the unit, blood typing, or how genetically similar a unit is to the recipient. Currently, unrelated donor units from Australia's cord blood banks are not consented for use for CP via SAS-Cat B. Therefore, autologous units from the public cord blood banks, or autologous or sibling units from Australia's private cord blood bank Cell Care, may be utilised for compassionate access.

Cell facility

Prior to infusion, the cord blood unit must be received from the cord blood bank, thawed, washed and prepared by a cell processing facility or lab using standard operating procedures. The cell facility plays an essential role in ensuring product quality, sterility, and preparation for recommended dosing under the guidance of clinicians.

For more general information about what CPA is doing to advance access to cord blood for CP, or to access resources for patients and families, please visit our website. If you wish to support a patient to receive treatment or want more information please contact us via celltherapies@cerebralpalsy.org.au

cerebralpalsy.org.au/advocacy/umbilical-cord-blood/

Selected references:

- Crompton, K., Godler, D. E., Ling, L., Elwood, N., Mechinaud-Heloury, F., Soosay Raj, T., Hsiao, K.-C., Fleming, J., Tiedemann, K., Novak, I., Fahey, M., Wang, X., Lee, K. J., Colditz, P. B., Edwards, P., & Reddihough, D. (2022). Umbilical Cord Blood Cell Clearance Post-Infusion in Immune-Competent Children with Cerebral Palsy. Cells Tissues Organs, 212(6), 546-553. https://doi.org/10.1159/000527612
- Finch-Edmondson, M., Paton, M. C. B., Webb, A., Ashrafi, M. R., Blatch-Williams, R. K., Cox, C. S., Crompton, K., Griffin, A. R., Kim, M. Y., Kosmach, S., Kurtzberg, J., Nouri, M., Suh, M. R., Sun, J., Zarrabi, M., Novak, I. (2025). Cord blood treatment for children with cerebral palsy: Individual participant data meta-analysis. Pediatrics, 155 (5): e2024068999. https://doi.org/10.1542/peds.2024-068999
- Finch-Edmondson, M., Paton, M. C. B., Honan, I., Karlsson, P., Stephenson, C., Chiu, D., Reedman, S., Griffin, A. R., Morgan, C., & Novak, I. (2022). Are We Getting It Right? A Scoping Review of Outcomes Reported in Cell Therapy Clinical Studies for Cerebral Palsy. J Clin Med, 11(24). https://doi.org/10.3390/jcm11247319
- Nguyen, T., Purcell, E., Smith, M. J., Penny, T. R., Paton, M. C. B., Zhou, L., Jenkin, G., Miller, S. L., McDonald, C. A., & Malhotra, A. (2023). Umbilical Cord Blood-Derived Cell Therapy for Perinatal Brain Injury: A Systematic Review & Meta-Analysis of Preclinical Studies. Int J Mol Sci, 24(5), 4351. https://doi.org/10.3390/ijms24054351
- Novak, I., Morgan, C., Fahey, M., Finch-Edmondson, M., Galea, C., Hines, A., Langdon, K., Namara, M. M., Paton, M. C., Popat, H., Shore, B., Khamis, A., Stanton, E., Finemore, O. P., Tricks, A., Te Velde, A., Dark, L., Morton, N., & Badawi, N. (2020). State of the Evidence Traffic Lights 2019: Systematic Review of Interventions for Preventing and Treating Children with Cerebral Palsy. Curr Neurol Neurosci Rep, 20(2), 3. https://doi.org/10.1007/s11910-020-1022-z
- Paton, M. C. B., Finch-Edmondson, M., Fahey, M. C., London, J., Badawi, N., & Novak, I. (2021). Fifteen years of human research using stem cells for cerebral palsy: A review of the research landscape. J Paediatr Child Health, 57(2), 295–296. https://doi.org/10.1111/jpc.15329
- Paton, M. C. B., Wall, D. A., Elwood, N., Chiang, K. Y., Cowie, G., Novak, I., & Finch-Edmondson, M. (2022). Safety of allogeneic umbilical cord blood infusions for the treatment of neurological conditions: a systematic review of clinical studies. Cytotherapy, 24(1), 2-9. https://doi.org/10.1016/j.jcyt.2021.07.001
- Sun, J. M., & Kurtzberg, J. (2021). Stem cell therapies in cerebral palsy and autism spectrum disorder. Dev Med Child Neurol, 63(5), 503–510. https://doi.org/10.1111/dmcn.14789
- Sun, J. M., Song, A. W., Case, L. E., Mikati, M. A., Gustafson, K. E., Simmons, R., Goldstein, R., Petry, J., McLaughlin, C., Waters-Pick, B., Chen, L. W., Wease, S., Blackwell, B., Worley, G., Troy, J., & Kurtzberg, J. (2017). Effect of Autologous Cord Blood Infusion on Motor Function and Brain Connectivity in Young Children with Cerebral Palsy: A Randomized, Placebo-Controlled Trial. Stem Cells Transl Med, 6(12), 2071-2078. https://doi.org/10.1002/sctm.17-0102