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# First approval for manufactured stem cell product

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**World's first approval for Prochymal, a manufactured stem cell product**

**Singapore:** In a first, Health Canada has approved marketing of Prochymal (remestemcel-L), a stem cell therapy by Osiris Therapeutics, for the treatment of acute graft-vs-host disease (GvHD) in children.

This is the world's first regulatory approval for a manufactured stem cell product and the first therapy approved for GvHD, which is a devastating complication of bone marrow transplantation that kills up to 80 percent of children affected.

"I am very proud of the leadership role Canada has taken in advancing stem cell therapy and particularly gratified that this historic decision benefits children who would otherwise have little hope," said Dr Andrew Daly, clinical associate professor, Department of Medicine and Oncology at the University of Calgary, Canada and principal investigator in the phase III clinical program for Prochymal. "As a result of Health Canada's comprehensive review, physicians now have an off-the-shelf stem cell therapy in their arsenal to fight GvHD."

Dr Daly said much like the introduction of antibiotics in the late 1920s, "with stem cells we have now officially taken the first step into this new paradigm of medicine".

Prochymal was authorized under Health Canada's Notice of Compliance with conditions pathway, which provides access to therapeutic products that address unmet medical conditions and which have demonstrated a favorable risk and benefit profile in clinical trials. Under the Notice of Compliance with conditions pathway, the sponsor must agree to carry out confirmatory clinical testing.

Health Canada's authorization was made following the recommendation of an independent expert advisory panel, commissioned to evaluate Prochymal's safety and efficacy. In Canada, Prochymal is now authorized for the management of acute GvHD in children who fail to respond to steroids. The approval was based on the results from clinical studies evaluating Prochymal in patients with severe refractory acute GvHD. Prochymal demonstrated a clinically meaningful response at 28 days post initiation of therapy in 61-64 percent of patients treated. The survival benefit was most pronounced in patients with the most severe forms of GvHD.

As a condition of approval, the clinical benefit of Prochymal will be further evaluated in a case matched confirmatory trial and all patients receiving Prochymal will be encouraged to participate in a registry that will monitor the long-term effects of the therapy.

Prochymal will be commercially available in Canada later this year. In addition to the extensive intellectual property protection Osiris has around Prochymal, which includes 48 issued patents, Health Canada's decision will also provide Prochymal with regulatory exclusivity within the territory. Canada affords eight years of exclusivity to Innovative Drugs such as Prochymal, and an additional six-month extension is available since it addresses a pediatric population.