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Australia announces world first trial for kids with Duchenne Muscular Dystrophy

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Three boys in New South Wales (NSW), Australia have become the youngest patients in the world to receive therapy for Duchenne Muscular Dystrophy (DMD) as part of a world-first international clinical trial for children aged under 4.

DMD is a rare and life-limiting genetic condition mostly affecting boys that causes rapid muscle weakness and results in almost all patients needing a wheelchair by 12 years of age. There is no known cure.

The trial will recruit 10 boys under 4 years of age worldwide, with patients followed over a period of at least 5 years to measure the therapy's effectiveness, including 3 in NSW who are being treated at The Children's Hospital at Westmead.

The DMD clinical trial will use a novel viral vector-based gene replacement therapy to target DMD at its root cause, replacing the faulty or mutated gene with a healthy version in a single-dose infusion.

Current management of DMD involves high-dosed steroids, combined with physical therapy and allied health support but while it can lead to some improvement, it is also associated with difficult side effects and is not a long-term treatment.

Gene replacement therapy has already shown success in treating other genetic conditions, including spinal muscular atrophy (SMA), a condition causing rapidly progressive muscle weakness and early death in children.

The DMD clinical trial was enabled by the Kids Advanced Therapeutics Programme at Sydney Children's Hospitals Network (SCHN), a programme supported by Luminesce Alliance and Sydney Children's Hospitals Foundation which aims to deliver clinical trials of advanced therapeutics and to speed up translation into clinical care.