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# Abu Dhabi plans to advance gene therapies for children with Spinal Muscular Atrophy Type 1

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**A best-in-class gene therapy contract development and research manufacturing organisation will be established**




The Department of Health – Abu Dhabi (DoH), the regulator of the healthcare sector in the Emirate, M42, a global health champion powered by artificial intelligence (AI), technology and genomics and PureHealth, the largest healthcare group in the Middle East, plan to partner with GEMMABio Therapeutics (GEMMABio), a pioneering global biotechnology company dedicated to advancing global access to gene therapies for rare diseases.

Through this strategic partnership, the entities will jointly establish dedicated manufacturing and research centres within Abu Dhabi's academic health institutions, enabling patients from across the MENA region to access cutting-edge clinical trials and, eventually, approved gene therapies.

The collaboration will kick off with a clinical trial evaluating a next-generation gene therapy product in children with spinal muscular atrophy type 1 (SMA1), setting a precedent for expanding access to life-saving therapies worldwide.

The collaboration will unite the shared vision and technology to further strengthen Abu Dhabi's gene therapy R&D ecosystem, which will serve as a regional hub for GEMMABio's translational and clinical research activities.

A best-in-class gene therapy contract development and research manufacturing organisation will be established in the Emirate as a joint venture between M42 and GEMMABio. Additionally, regional access to GEMMABio's commercialised gene products will be coordinated through Abu Dhabi's gene therapy research and treatment centres.

Supported by the Emirate's state-of-the-art infrastructure, forward-looking policies and the Health, Endurance, Longevity and Medicine (HELM) Life Science Cluster, Abu Dhabi is offering streamlined licensing and co-commercialisation processes, alongside co-investment schemes and tax relief for biotech startups.

This strategic partnership reinforces Abu Dhabi's recent strides in rare disease gene therapies and precision medicine. The Emirate has significantly expanded its genomics capabilities through the Emirati Genome Programme, with over 800,000

genome samples sequenced, marking one of the world’s most advanced national genomic databases.