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## Acuitas' CSO Co-Authors Article in *Molecular Therapy* With Scientists From Children's Hospital of Philadelphia and Perelman School of Medicine

Vancouver, B.C. – Acuitas' Chief Scientific Officer, Dr. Ying Tam, collaborated with scientists from the Children's Hospital of Philadelphia and Perelman School of Medicine to determine whether messenger RNA lipid nanoparticles (LNP) can improve outcomes of hematopoietic stem cells (HSCs) in the context of Fanconi anemia (FA).

Their studies demonstrate that LNP efficiently deliver messenger RNA (mRNA) to murine bone marrow HSCs after either intravenous or intrafemoral injection of LNP. LNP-mediated delivery of *Fancc* mRNA restored hallmark functions of FA hematopoietic stem and progenitor cells in a murine FANCC-deficient model both *in vitro* and *in vivo*, including ex vivo expansion and resistance to alkylating agents. These data support further development of this approach for potential clinical therapies for FA patients. (*Molecular Therapy* 2025)

Please click <u>here</u> to read the publication.

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