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Targeted therapy for fibrotic remodeling in LMNA-related dilated cardiomyopathy

Stanford researchers have developed an innovative, non-invasive therapeutic strategy to treat myocardial fibrosis (MF), a key driver of arrhythmia and heart failure in LMNA-related dilated cardiomyopathy (LMNA-DCM).

LMNA-DCM is a genetic form of cardiomyopathy caused by mutations in the LMNA gene and is characterized by early-onset myocardial fibrosis, conduction system disease, malignant arrhythmias, and progressive heart failure. Current therapies are largely supportive and do not target the root causes of fibrosis, which often occurs independently of left ventricular dilation. A major limitation of existing treatments is their inability to address fibrosis driven by mechanisms such as endothelial dysfunction and endothelial-to-mesenchymal transition (EndoMT).

To overcome this gap, Stanford researchers investigated the molecular and cellular basis of fibrosis in LMNA-DCM using patient-derived heart tissue, 3D cardiac organoids, and animal models. They identified a gene-driven fibrotic pathway that operates independently of traditional neurohormonal mechanisms. Pharmacological modulation of this pathway successfully prevented fibrotic remodeling, preserved endothelial identity, and improved cardiac function in preclinical models.

This approach establishes a novel, scalable therapeutic platform that targets the underlying mechanisms of fibrosis and offers a promising path toward disease-modifying treatments for LMNA-DCM and other fibrotic heart conditions.

Stage of Development
Research - in vivo

Applications

- Therapeutic therapy for LMNA-related dilated cardiomyopathy
- Disease-modifying treatment for fibrotic heart conditions of genetic or non-genetic origin

Advantages

- First-in-class therapeutic targeting gene-driven cardiac fibrosis
- Non-invasive and scalable therapeutic approach
- Preserves cardiac conduction and reduces arrhythmia risk in preclinical models
- Demonstrated efficacy in patient-derived tissues, 3D organoids, and in vivo models

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