Docket #: S18-305

Therapeutic targeting of the Platelet-Derived Growth Factor (PDGF) signaling pathway in dilated cardiomyopathy

Dilated cardiomyopathy (DCM) is principally characterized by left ventricular enlargement and/or a reduction in systolic function. In many cases for individuals with DCM, no etiology can be determined, therefore clinicians should suspect that a pathogenic variant of the LMNA gene may be the underlying cause. Initial signs of LMNA-related DCM are sudden cardiac arrest leading to death, owing to prevalence of arrythmias in the individual. Researchers at Stanford University have developed a novel approach to the treatment and prevention of proarrhythmic in LMNA-related DCM by establishing the link between hyperactivation of signaling pathways and the LMNA gene in patient-specific induced pluripotent stem cell-derived cardiomyocytes (iPSC-CMs), and subsequent treatment using tyrosine kinase inhibitors.

Applications

- Treatment and prevention of LMNA-related dilated cardiomyopathy

Advantages

- Reduces the risk of cardiac arrest and sudden death
- Systemic, non-invasive, therapies to treat DCM using tyrosine kinase inhibitors

Publications

- Lee, J., Termglinchan, V., Diecke, S., Itzhaki, I., Lam, C. K., Garg, P., . . . Wu, J. C. Activation of PDGF pathway links LMNA mutation to dilated cardiomyopathy *Nature* August 2019.

Patents

- Published Application: 20230032239

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