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Lonza collaborates with DiNAQOR AG to advance gene therapy programs

27 November 2019 | News

Strategic collaboration to focus on DiNAQOR's adeno-associated virus gene therapy production system

Lonza and DiNAQOR AG, a global gene therapy platform company, have announced a strategic collaboration to advance DiNAQOR's preclinical programs for the treatment of cardiac myosin-binding protein-C (MYBPC3) cardiomyopathies, a genetic condition that can result in heart failure.

Under the terms of the collaboration, Lonza will provide DiNAQOR preclinical, clinical and commercial production support for the company's lead preclinical program DiNA-001, an adeno-associated virus (AAV) gene therapy program for patients with MYBPC3-linked cardiomyopathy**.** Lonza's state-of-the-art cell-and-gene-therapy manufacturing facility in Houston, TX (US) will handle all product supply for DiNA-001.

Through this partnership, DiNAQOR will be able to leverage Lonza's extensive, dedicated teams and laboratories for viral-vector process-development, located in Houston (US).

In addition to its cardiac gene therapy platform, DiNAQOR is also developing a local-regional delivery system for the heart. This will allow the company to route gene therapy directly to the cardiac muscle maximizing biodistribution and transduction of the cardiomyocytes. This approach will look to minimize potential adverse effects of systemic gene therapy delivery.

Cardiomyopathy is a disease of the heart muscle that can lead to heart failure. Approximately 50% of all cardiomyopathies are caused by a single-gene or monogenic defect. There are 1.7 million people in the European Union and the United States currently affected by a monogenic cardiomyopathy, 300,000 of these individuals have a defect in the MYBPC3 gene. There is currently no cure for patients living with genetic cardiomyopathies.