

## **BioMarin and Skyline Therapeutics Announce Strategic Collaboration to Develop Novel Gene Therapies for Cardiovascular Diseases**

SAN RAFAEL, Calif. and SHANGHAI, Dec. 16, 2021 [/PRNewswire/](#) -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) and Skyline Therapeutics (formerly Geneception), a gene and cell therapy company focused on developing novel treatments for unmet medical needs, today announced a multi-year global strategic collaboration for the discovery, development and commercialization of Adeno-Associated Virus (AAV) gene therapies to treat genetic cardiovascular diseases.

The partnership will leverage Skyline Therapeutics' integrated AAV gene therapy platform based on its proprietary vector engineering and design technology and manufacturing capability to develop innovative gene therapies with a focus on genetic dilated cardiomyopathies (DCM), a group of progressively advancing, devastating diseases with no targeted treatment options.

Under the agreement, BioMarin and Skyline Therapeutics will collaborate on discovery and research through to an Investigational New Drug Application (IND). BioMarin brings experience in gene therapy development, cardiovascular biology and insights into genetic basis of diseases, and Skyline contributes its expertise in developing gene therapy products including vector engineering and design technology and manufacturing capabilities to this collaboration. Each company will advance the programs through clinical development in their pre-defined territories.

In support of its R&D efforts for the collaborative projects, Skyline Therapeutics will receive an undisclosed payment associated with signing, comprising an upfront payment and an equity investment from BioMarin, and is eligible to receive pre-specified payments for R&D, regulatory and commercial milestones.

BioMarin will have the rights to commercialize therapeutic products resulting from the collaboration in its territories, including the United States, Europe, and Latin America, and Skyline Therapeutics will be responsible for commercialization in the Asia-Pacific region. In addition, Skyline Therapeutics will be eligible to receive royalty payments on future sales from BioMarin in its territories.

"We are thrilled to announce what we anticipate will be a fruitful collaboration at the interface between Skyline's innovative approach to AAV vector engineering and design

and our team's proven expertise in creating and developing gene therapies," said Kevin Eggan, Group Vice President, Head of Research and Early Development, from BioMarin.

"We are excited to partner with Skyline Therapeutics to tackle these genetic forms of dilated cardiomyopathy. This collaboration strengthens our leadership in cardiac gene therapy and extends our R&D collaboration to Asia, where a large number of patients suffer from these devastating diseases," said Brinda Balakrishnan, Group Vice President, Corporate and Business Development at BioMarin. "We look forward to fostering this collaboration and bringing transformative medicines to patients worldwide."

"Dilated cardiomyopathy is a serious cardiac disorder in which structural or functional abnormalities of the heart muscle can lead to complications such as arrhythmia and heart failure, resulting in substantial morbidity and mortality. Mutations in many genes are associated with the development of DCM, among other etiologies for the disease," said Jay Hou, Chief Scientific Officer at Skyline Therapeutics. "Together with BioMarin's team we have identified a number of critical genes associated with DCM. We are delighted to work closely with BioMarin and apply our AAV vector technology to interrogate these new targets and develop novel treatments for DCM patients."

"The collaboration with BioMarin leverages both companies' capabilities in the development of gene therapies. With the BioMarin team, we share the goal of working in concert to develop therapies for genetic cardiovascular disease that address high unmet medical needs," said Amber Cai, CEO of Skyline Therapeutics. "Together, we will utilize gene therapy to tackle cardiovascular diseases with a disease modifying trailblazing approach that could change the treatment paradigm in these conditions."

### **About Dilated Cardiomyopathy (DCM)**

DCM is a common cause of heart failure and end-stage DCM, which often leads to heart transplantation. Despite improvements in pharmacotherapy and care, the five-year survival rate of DCM is only about 50%. Hundreds of thousands of patients suffer from the genetic forms of DCM in U.S., EU, China, and Japan. More than 50 genes associated with DCM have been identified, accounting for 40-50% of familial DCM cases. Many of these genes encode proteins with important known functions in cardiomyocytes related to cytoskeletal, sarcomere and nuclear envelope biology. Our aim is to correct the pathways altered by these genetic contributors to DCM through AAV based gene therapy, in each case addressing the root cause of the disease.

### **About BioMarin**

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare genetic diseases. The company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.biomarin.com](http://www.biomarin.com). Information on such website is not incorporated by reference into this press release.

### **About Skyline Therapeutics**

Skyline Therapeutics is a fully integrated gene and cell therapy company dedicated to the discovery, development and delivery of innovative therapies. Established in 2019, Skyline Therapeutics has built a proprietary AAV-based gene therapy platform that integrates novel capsid engineering and vector design, analytical and process development, and state-of-the-art GMP manufacturing capabilities that support large scale clinical-grade vector production. The Skyline team of world-class experts and leaders in science, technology and business brings industry-leading know-how and is advancing a pipeline of diversified programs that address multiple diseases including ocular, neurological, metabolic and blood disorders. Skyline Therapeutics is also broadening its therapeutic expertise to cover more disease areas with high unmet need such as cardiovascular disorders through strategic partnerships. Headquartered in China, Skyline Therapeutics currently has research, development and manufacturing capabilities in Shanghai and Hangzhou. [www.skytx.com](http://www.skytx.com)

### **Forward-Looking Statements**

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: expectations related to the multi-year global strategic collaboration with Skyline for the discovery, development and commercialization of AAV gene therapies for dilated cardiomyopathy and pre-specified payments to Skyline for R&D, regulatory and commercial milestones, and the rights to commercialize therapeutic products resulting from the collaboration in its territories, including the United States, Europe, and Latin America. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. Additional important factors to be considered in connection with forward-looking statements are detailed from time to time under the caption "Risk Factors" and elsewhere in BioMarin's Securities and Exchange Commission (SEC) filings, including BioMarin's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, and future filings and reports by BioMarin. BioMarin undertakes no duty or obligation to update any

forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc.

## References

1. Givertz MM, Mann DL. Epidemiology and natural history of recovery of left ventricular function in recent onset dilated cardiomyopathies. *Curr Heart Fail Rep*. 2013;10(4):321-330. doi:10.1007/s11897-013-0157-5
2. Bozkurt B, Colvin M, Cook J, et al. Current Diagnostic and Treatment Strategies for Specific Dilated Cardiomyopathies: A Scientific Statement From the American Heart Association [published correction appears in *Circulation*. 2016 Dec 6;134(23):e652]. *Circulation*. 2016;134(23):e579-e646. doi:10.1161/CIR.0000000000000455
3. Prevalence of rare diseases: Bibliographic data, Orphanet Report Series, Rare Diseases collection, January 2021, Number 1: Diseases listed in alphabetical order.  
[http://www.orpha.net/orphacom/cahiers/docs/GB/Prevalence\\_of\\_rare\\_diseases\\_by\\_diseases.pdf](http://www.orpha.net/orphacom/cahiers/docs/GB/Prevalence_of_rare_diseases_by_diseases.pdf)
4. Miura K, Nakagawa H, Morikawa Y, et al. Epidemiology of idiopathic cardiomyopathy in Japan: results from a nationwide survey. *Heart*. 2002;87(2):126-130. doi:10.1136/heart.87.2.126
5. Huang GY, Gao H, Meng XG. Epidemiology and etiology of dilated cardiomyopathy. *Chinese Journal of Prevention and Control of Chronic Diseases*. 2009;17(1):42-44.
6. Jing Zhong, Li-Ping Li, Jian-Feng Zhou and Yong-He Ding (November 5th 2020). Genetic Determinant of Familial Dilated Cardiomyopathy and Genotype-Targeted Therapeutic Strategy, *Cardiac Diseases - Novel Aspects of Cardiac Risk, Cardiorenal Pathology and Cardiac Interventions*, David C. Gaze and Aleksandar Kibel, IntechOpen, DOI: 10.5772/intechopen.94434. Available from:  
<https://www.intechopen.com/chapters/73939>

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