

## **BMS, uniQure collaborate to develop gene therapies for CVDs**

07 April 2015 | News | By BioSpectrum Bureau

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The companies anticipate the collaboration to be effective during the second quarter of 2015.

The collaboration includes uniQure's proprietary gene therapy program for congestive heart failure that is intended to restore the heart's ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and thereby improve clinical outcomes for patients with reduced ejection fraction.

Beyond cardiovascular diseases, the agreement also includes the potential for target-exclusive collaboration in other disease areas.

In total, the companies may collaborate on ten targets, including S100A1.

uniQure will lead discovery efforts and be responsible for manufacturing of clinical and commercial supplies using its vector technologies and its industrial, proprietary insect-cell based manufacturing platform.

BMS will be solely responsible for commercialization of all products from the collaboration.

Mr Joern Aldag, CEO, uniQure, said, "This collaboration will accelerate the application of gene therapy for large patient populations suffering from heart diseases and will complement the further development of uniQure's internal pipeline in two focus areas: liver diseases, including hemophilia, and CNS, including lysosomal storage diseases."

Under the terms of the agreement, Bristol-Myers Squibb will make near-term payments of approximately \$100 million, including an upfront payment of \$50 million to be made at the closing of the transaction, a \$15 million payment for the selection of three collaboration targets, in addition to S100A1, to be made within three months of the closing; and an initial equity investment in uniQure for a number of shares that will equal 4.9 percent of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or at least \$32 million in total.

BMS will acquire an additional 5.0 percent ownership before December 31, 2015, at a 10 percent premium, and will be granted two warrants to acquire up to an additional 10 percent equity interest, at a premium, based on additional targets being introduced into the collaboration.

The parties have also agreed to enter into a supply contract, under which uniQure will undertake manufacturing of all gene therapy products under the collaboration.

uniQure will be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead S100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration.

The company is also eligible to receive net sales based milestone payments and tiered single to double-digit royalties on product sales.