

Success story

BMS AND UNIQUIRE CLOSED THE BIGGEST GENETHERAPY DEAL SO FAR.

ulbrich&partner acted as business advisor.

> Challenge

uniQure is a leader in gene therapy with the most advanced fully integrated technology platform and multiple pipeline programs in CNS, liver and cardiovascular diseases.

Several pharma companies competed against each other wanting to cooperate with uniQure on the development

of its cardiovascular pipeline. uniQure is focused on developing a proprietary pipeline in the rare / orphan disease space and was willing to enter in a strategic alliance for treatments of common diseases for larger populations such as congestive heart failure.

"It is immensely exciting to see the potential of our initial discoveries recognized first by uniQure and then advanced to a stage where we can build a portfolio of gene therapies to treat cardiovascular disease in partnership with Bristol-Myers Squibb,"

"I would like to thank Dr. Claudia Ulbrich for effectively working on this important transaction that has contributed to bringing InoCor closer to helping patients."

Prof. Patrick Most, Managing Director of uniQure Germany

> Background / Solution

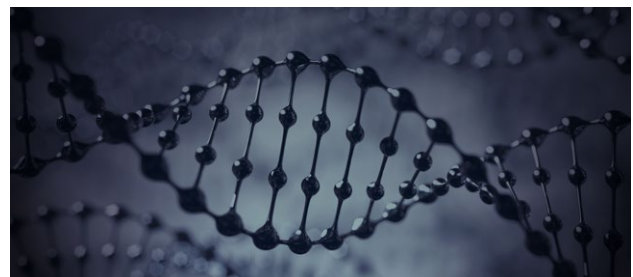
uniQure's cardiovascular program, led by S100A1, appeared to be a very attractive area for a multi target collaboration with a strategic partner.

S100A1 as a calcium sensor and master regulator of heart function represents a promising and disruptive curative gene therapy to treat chronic heart failure, a multi bn dollar market with 20 Mio affected people worldwide.

S100A1 gene therapy demonstrated an increase in survival rates after myocardial infarction-induced heart failure in a human relevant pig model by 90%.

The program was acquired 2014 through a 100% acquisition of InoCard GmbH, a German spin-off company of the University Hospital of Heidelberg, founded by Prof. Patrick Most and Prof. Hugo Katus. ulbrich& partner acted as business advisor

for the founders in setting up InoCard GmbH. In her function as Interim CEO and business advisor, Dr. Claudia Ulbrich orchestrated the sale of InoCard GmbH successfully to uniQure N.V., only 6 months after the inception of the company. ulbrich&partner began their assignment with the company in October 2014, a month, in which the founder team won the first prize of the Health Axis Accelerator business plan competition in Heidelberg.



Through the acquisition InoCard secured R&D funding as well as technical, regulatory and manufacturing expertise from uniQure.

"The leading role of Dr. Ulbrich was key to success and cornerstone for the future oriented deal with uniQure. Dr. Ulbrich identified uniQure as the best possible partner for InoCard and seamlessly managed as interim CEO the merger and integration of our disruptive technology."

"It would not have happened without Claudia."

Prof. Patrick Most, Managing Director of uniQure Germany

> Approach

uniQure asked Claudia Ulbrich to work with its CBO Hans Preusting and lead the partnering of the cardiovascular business unit. After initial scientific discussions with various partners, uniQure received several non-binding term sheets in January of 2014, allowed pharma partners to enter into a competitive diligence procedure and start contract negotiations in parallel.

These resulted in closing a broad license agreement, a supply agreement and an equity agreement within three months of starting the diligence and negotiation process.

ulbrich&partner coordinated all business related negotiations. An international law firm provided legal support.

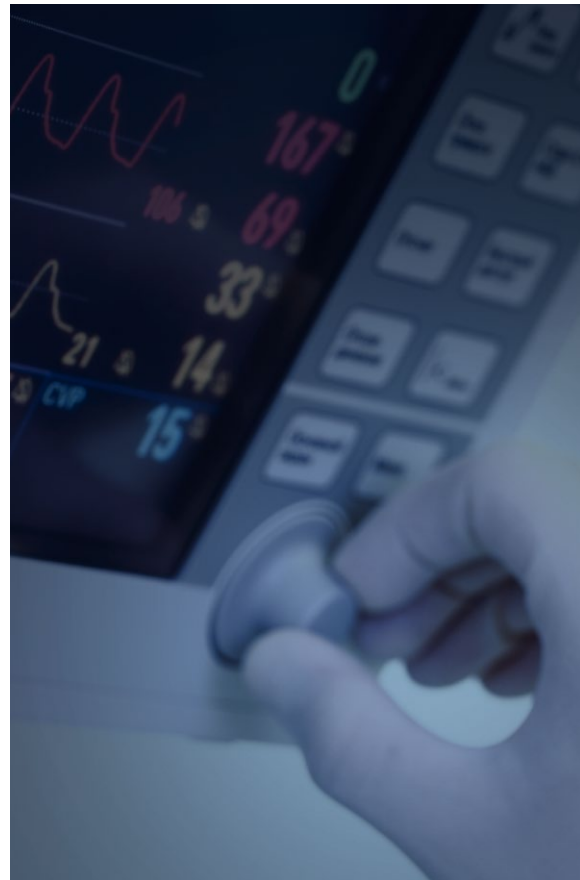
Results

The parties collaborate to develop S100A1 gene therapy and 9 additional programs on cardiovascular and other targets. BMS gained exclusive access to uniQure's gene technology / platform for cardiovascular targets.

In return, uniQure received more than 100 m U.S. dollar in near term payments, including an upfront payment of 50m U.S. dollar and an initial equity investment representing 4.9% of the total number of shares at a purchase price of 33.84 U.S. dollar or approximately 37M U.S. dollar in total. BMS has the right to acquire further equity of 5.0% at a premium.

In addition, uniQure granted BMS warrants to acquire an additional 10% equity at a premium conditioned upon progress of the collaboration.

BMS will fund all R&D and uniQure may receive R&D milestones per target ranging from 217M U.S. dollar to 254m U.S. dollar for the lead compound. In addition, uniQure may receive tiered up to double-digit royalties. The parties have also agreed to enter into a supply contract, under which uniQure will undertake to manufacture all commercial supply for gene therapy products.



The total potential consideration represents a value of more than 2.3bn U.S. dollar, the largest deal in the history of gene therapy so far.

"Bristol-Myers Squibb's strength in the cardiovascular field and its commitment to gene therapy will allow them to leverage the full breadth and capacity of our platform for cardiovascular diseases. In addition to working closely with Bristol-Myers Squibb in cardiovascular disease, this collaboration will enable uniQure to progress its own proprietary portfolio in gene therapy programs, most notably its Hemophilia B and its Sanfilippo B programs,"

„Thank you, Claudia, for all your important contributions!“

Joern Aldag, Chief Executive Officer of uniQure

Profiles

ulbrich&partner

Management Consulting

Location:

Hannover, Germany

Company:

Consulting for management, transaction, finance, strategy, lifescience and business development

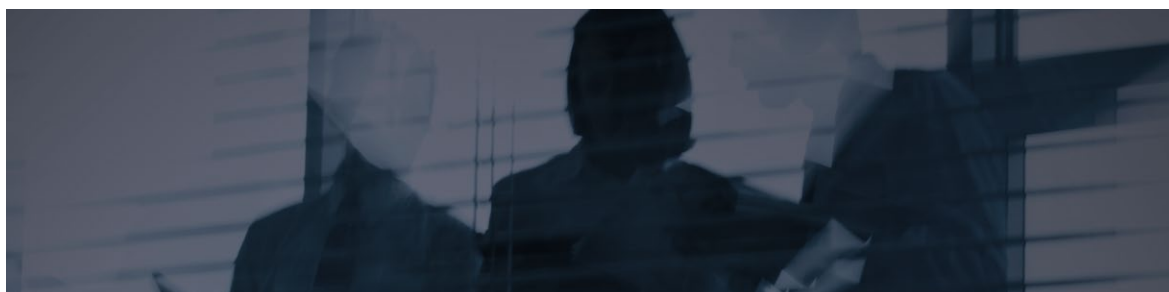
uniQure

Location:

Amsterdam, Netherland

Company:

uniQure is a leader in the field of gene therapy and had developed the first and currently the only gene therapy product to receive regulatory approval in the European Union



Contacts



Dr. Claudia Ulbrich

ulbrich&partner Management Consulting
Güntherstrasse 7A | 30519 Hannover | Germany
Phone +49 (0) 511 / 37 48 40 51
Mobile +49 (0) 172 / 455 76 32
cu@ulbrichpartner.com
www.ulbrichpartner.com



Joern Aldag

Chief Executive Officer uniQure N.V.
Meibergdreef 61 | 1105 BA | Amsterdam | Netherlands
Phone +31 (0) 20 / 420 60 01
j.aldag@uniquire.com
www.uniquire.com



Prof. Dr. Patrick Most

Managing Director uniQure GmbH
Im Neuenheimer Feld 410 | 69120 Heidelberg | Germany
Phone +49 (0) 6221 / 56 78 43
Mobile +49 (0) 174 / 990 08 67
p.most@uniQure.com
www.uniquire.com