

# Big Hopes with Small Molecules – PIQUR Therapeutics AG is aiming to Turn Cancer into a Manageable Disease

PIQUR Therapeutics AG\*

**Abstract:** The origins of PIQUR Therapeutics AG, which was established in 2011, are found in significant research work at the University of Basel. The main focus of the spin-off of the University of Basel is the active pharmaceutical ingredient PQR309, which was used for the first time at the beginning of 2014 in Phase I trials in humans at University Hospital Basel. The small molecule, which was developed for targeted cancer therapy, intervenes with two major signaling pathways of cell growth. In just three years PIQUR secured funding of over 37 million Swiss Francs from private investors and Versant Venture, a leading venture capital firm.

**Keywords:** Basel · Cancer therapy · Phase I trials · Small molecule · Spin-off

The main focus of research and development at PIQUR is the inhibition of two signaling pathways of cell growth: firstly, inhibition of the important  $PI_3$  kinase (phosphoinositide 3-kinase) and, secondly, inhibition of mTOR (mammalian target of rapamycin), a further major hub for cell growth. PIQUR's CEO and Co-Founder Vladimir Cmiljanovic, who came to Basel as a professional Serbian handball player more than ten years ago, got into phosphoinositide 3-kinase (PI3K) research at the end of his Bachelor degree. Subsequently he worked on chemical antibodies and identified the possibility to create a drug for treating cancer. He followed up on this idea with his PhD thesis and managed to secure a grant from Novartis and the University of Basel to do the proof of concept on mice. Pursuing this line of research in a post-doctoral fellowship, he reached the crossroad in his career to either follow an academic path or to start his own company. Having learned about entrepreneurship from the cradle, being born to a father who supplied the automo-

tive industry with chemicals, Cmiljanovic chose the entrepreneurial route. Jointly with Professor Matthias Wymann, University of Basel and Basel Emeritus Professor Bernd Giese, Chairman of the Board of Directors of PIQUR today, he founded Piqu Therapeutics AG in 2011. Wymann, Professor at the Department of Biomedicine of the University of Basel and an expert in  $PI_3$  kinases, is involved in several research projects looking into cellular processes of cancer development and inflammation. Particular focus is placed on cell receptor-coupled enzymes that control cell growth and cell migration. Known as lipid kinases, these enzymes change the internal surface of the cell membrane and are often hyper activated in the case of disease. If these processes are out of balance, oncolipids may develop. These oncolipids activate signaling pathways that can transform a completely normal body cell into a tumor site. The cell divides in an uncontrolled manner, immunizes itself against the body's own defense mechanisms, and starts to spread through the whole body.

## Entrepreneurial Spirit and a Pragmatic Approach in Solving Problems is Essential

Cmiljanovic played an instrumental role in setting up the company and the management team. From the early days Cmiljanovic was supported by Andreas Emmenger, Co-Founder, Member of the Board of Directors of PIQUR and CFO at Molecular Partners, as well as Ralf Rosenow, legal adviser of PIQUR, attorney-at-law and partner of the law firm Blum&Grob Attorneys at Law. "Beside robust clinical data you need to attract the right mix of talents covering a broad range of expertise in science and business", Cmiljanovic commented. And added: "Colleagues must bring in an entrepreneurial spirit and a pragmatic approach in solving problems as building up a pharmaceutical company from scratch involves many challenges and risks." The third element of equal importance is, according to Cmiljanovic, to attract investors who trust the management and share the strategy and vision of the company. "They must be ready to take the inherent risks linked to the development of new and innovative therapies", commented Cmiljanovic further.

Shortly after the incorporation of the company Cmiljanovic was able to recruit the Basel Emeritus Professor of Oncology, Richard Herrmann, as Chief Medical Officer and entrepreneur Andreas Emmenegger, CFO of Molecular Partners, as a Member of the Board of Directors with industry expertise. A number of industry-experienced specialists joined PIQUR over time. Dorian Fabbro, a former Novartis scientist, who significantly contributed to



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the development of a number of Novartis' blockbusters, joined the company as Chief Scientific Officer. Dorianio has contributed to the discovery and development of various protein kinase inhibitors of Novartis for the treatment of cancer; e.g. Midostaurin<sup>®</sup>, Glivec<sup>®</sup>, Afinitor<sup>®</sup>, and Tasigna<sup>®</sup>. Frances Betts acts as PIQUR's Chief Development Officer and has over 20 years of clinical development experience in Big Pharma as well as in Biotech start-ups. At Roche she contributed to successful submissions of products like Roferon<sup>®</sup>, Neupogen<sup>®</sup> and MabThera<sup>®</sup>. The Scientific Advisory Board of PIQUR can rely on the support of mTOR pioneer Professor Michael N. Hall of the Biozentrum. The University of Basel supported the company from the outset, co-financed the patent registration, and provides the researchers with a laboratory and office space.

### Since Incorporation PIQUR Has Raised 37 million Swiss Francs

In the first phase PIQUR was primarily financed by private investors, but has also received substantial non-dilutive contributions from the Swiss Innovation Promotion Agency CTI and from the Eurostars program, a joint European initiative for small- and medium-sized research companies.

In April this year Versant Ventures, a leading venture capital firm that specializes in investments in innovative biopharmaceuticals, medical devices and other life science opportunities, invested 11 million Swiss Francs in PIQUR. Since the start of the company PIQUR has raised total proceeds of over 37 million Swiss Francs. Concurrent with the investment by Versant, Guido Magni, Venture Partner with Versant Ventures in Basel, has joined PIQUR's Board of Directors. He previously was the Head of the Medical Science Department of Roche Pharmaceuticals and oversaw the development and the registra-

tion of a large number of key oncology drugs. In addition, Gianni Gromo, Venture Partner with Versant Ventures, will act as an observer in PIQUR's Board of Directors and will give strategic advice to the company. He was previously the Global Head of the Cardio Metabolic Disease Area at Roche Pharmaceuticals.

### Phase I Results Expected in the Fourth Quarter 2014

PIQUR uses the funds primarily for clinical trials with the most advanced active ingredient PQR309. The aim of the trials that started in January 2014 is to investigate the safety and tolerability of PQR309 in humans and to determine the ideal dose. First trial results are expected in the fourth quarter of 2014 – a significant milestone for this pharmaceutical company that is only three years old. The company envisions bringing its first cancer therapy to market in 2017.

Received: September 16, 2014

“ “ For the discovery and development of innovative medicines and diagnostic solutions, collaboration between academic institutions, medical community and industry is absolutely crucial. An important element therefore is a vibrant environment for venture capital and start-ups. Switzerland still needs to catch up with the big hubs on the West and East coast of the United States. In this regard, I have sensed very positive momentum over the last couple of years. ” ”

*Severin Schwan, CEO Roche Group*