

Media Release

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PIQUR announces positive Phase 1 study results and the start of Phase 2 of PQR309

Study confirms that PQR309 is well tolerated and shows promising signs of clinical antitumor activity

PIQUR Therapeutics AG, a Swiss clinical-stage pharmaceutical company, today announced positive results from the Phase 1 first-in-human study with PQR309 in advanced solid tumors.

PQR309 is a novel, balanced pan-PI3K/mTOR inhibitor formulated for oral administration. Preclinical studies demonstrated that PQR309 is a highly selective kinase inhibitor, penetrating blood-brain barrier with potent *in vitro* as well as *in vivo* antitumor activity.

The results from the first-in-man Phase 1 trial indicate that PQR309 is safe and well tolerated with expected pharmacokinetic profile, pharmacodynamic effect as well as clinical activity.

In total, 28 patients with advanced solid tumors were treated in cohorts of escalating PQR309 doses. The maximum tolerated dose (MTD) of PQR309 was determined to be 80 mg administered at continuous once-daily dosing schedule. Pharmacokinetic data indicate fast absorption of PQR309 as well as dose proportionality. Pharmacodynamic assessment in tumors shows downregulation of PI3K/mTOR pathway phosphoproteins.

One patient with a dysregulation of the mTOR-pathway demonstrated a confirmed partial response while another patient with a tumor containing a PI3K mutation, showed important tumor reduction and a minor response according to RECIST (Response Evaluation Criteria in Solid Tumors). “Data obtained from this first-in-human clinical study, including the evidence of clinical activity is very encouraging and highlights the clinical potential of PQR309”, commented Dr. Sasa Dimitrijevic, Chief Development Officer of PIQUR.

The results of this study will be presented at ASCO, May 29 to June 2, 2015:

[“A phase 1 first-in-human \(FIH\) dose-escalation \(DE\) study of the oral dual PI3K/mTOR inhibitor PQR309 in patients \(pts\) with advanced solid tumors: Final DE results.”](#) [Poster Session: Developmental Therapeutics—Clinical Pharmacology and Experimental Therapeutics, Sub-category: Signal Transduction, Abstract Number 2592, Mon, May 30, 8:00 - 11:30 AM, Location: S Hall A, Poster Board #308]

PIQUR has also received approval from the health authorities for the initiation of a Phase 2 clinical trial with PQR309, which aims to obtain efficacy data at well-tolerated doses in patients with selected solid tumor and hematological indications with aberrant PI3K/mTOR signaling. “Following recent regulatory approval to start a Phase 2 study of PQR309, we are excited to advance our compound as a potential treatment option for selected cancer indications”, commented Prof. Dr. Richard Herrmann, PIQUR’s Chief Medical Officer.

Helping patients to survive cancer

PIQUR aims to help patients to survive cancer. Two out of three people are now living at least five years after their cancer has been diagnosed. Despite of significant medical innovations in the treatment of cancer, there remains a high unmet medical need for therapies that prolong patients’ survival and improve their quality of life. PIQUR targets both PI3K (phosphoinositide 3-kinase) and mTOR (mammalian target of rapamycin), two key signaling molecules that are vital to several essential biological processes involved in malignant disease, such as cell proliferation, survival and metastasis, making them attractive targets in cancer therapy.

About PQR309

PIQUR’s lead compound, PQR309, is a novel, oral, balanced pan-class 1 PI3K/mTOR inhibitor with excellent prospects to become a powerful anti-cancer drug. PQR309 compares favorably to current and clinically most advanced pan-PI3K/mTOR inhibitors with respect to the drug-like properties. Unlike most of its competitors, PQR309 crosses the blood-brain barrier, expanding its use to oncologic as well as hematologic malignant diseases involving the brain. PQR309 showed activity in different aggressive cancer cell lines inhibiting the PI3K/mTOR pathway.

About PIQUR

PIQUR is a Swiss clinical-stage pharmaceutical company incorporated in August 2011 as a spin-off of the University of Basel, focusing on the discovery and development of innovative anti-cancer drugs based on lipid kinase (PI3K) and mTOR inhibition. PIQUR’s pipeline originates from one of the most promising research areas in oncology. Both PI3K and mTOR are clinically validated drug targets in oncology. PIQUR has a secured patent scope protecting many chemical compounds.
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