PIQUR
TARGETED CANCER THERAPIES

Media Release

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PIQUR Receives Orphan Drug Designation from FDA for PQR309 in PCNSL

PIQUR Therapeutics AG, a Swiss clinical-stage pharmaceutical company, announced today that the U.S. Food & Drug Administration (FDA) has granted orphan drug designation to PIQUR's lead compound PQR309 for the treatment of primary central nervous system lymphoma (PCNSL).

PCNSL is a very rare and aggressive form of lymphoma involving brain and its linings, eyes or spinal cord. The disease affects less than 1 person in 100,000 in the USA with approximately 7,500 new cases reported annually in the USA, Europe and Japan. Treatment options available for PCNSL are limited, and the prognosis with current therapies is poor.

"We are very pleased to receive FDA orphan drug designation for PQR309 in PCNSL. This is an important regulatory milestone for the company and a significant step towards the clinical advancement of PQR309," said Dr. Ruggero Della Bitta, Chief Medical Officer of PIQUR. "This represents an important step towards addressing a high unmet medical need and bringing a potential treatment to those with this rare and life-threatening disease."

The Orphan Drug Designation Program, administered by the FDA's Office of Orphan Products Development, is intended to encourage companies to develop therapeutics for diseases that affect fewer than 200,000 people in the USA. The designation provides the company several benefits and incentives, including assistance with clinical study design and drug development, tax credits for qualified clinical trials costs, exemptions from certain FDA application fees, as well as a seven-year period of market exclusivity upon regulatory product approval.

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Notes to Editors

Contacts

Media Relations:
Tatsuo Satoh
Head IT & Communications
T: +41 61 633 29 38
M: +41 78 819 17 44
tatsuo.satoh@piqur.com

Investor Relations:
Gaudenz von Capeller
Chief Financial Officer
T +41 61 633 29 31
M: +41 79 798 64 43
gaudenz.capeller@piqur.com

About PQR309

PIQUR's lead compound, PQR309, is an oral, brain-penetrant, dual inhibitor of the PI3K/mTOR pathway, which is activated in 60 – 80% of human cancers. Unlike most of its competitors, PQR309 crosses the blood-brain barrier, expanding its use to malignant diseases involving the brain. Preclinical and Phase 1 studies have shown PQR309 to have a favorable safety, tolerability and pharmacokinetic profile. In addition, PQR309 has shown both preclinical activity in various tumor models and clinical activity in Phase 1 and 2 studies.

PQR309 is currently being investigated in five Phase 1 and 2 clinical studies in advanced solid tumors (NCT02483858), relapsed or refractory lymphoma (NCT02249429), relapsed or refractory PCNSL (NCT02669511) and progressive glioblastoma multiforme (NCT02850744). In addition, the PIQHASSO Phase 1/2b study investigates PQR309 in combination with Eisai's Eribulin in metastatic HER2-negative and triple-negative breast cancer (NCT02723877).

About PIQUR Therapeutics

PIQUR Therapeutics 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 the inhibition of lipid kinase (PI3K) and mTOR. 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 holds a worldwide exclusive license on the intellectual property rights for PQR309 and related series of analogues, for development and commercialization. www.piqur.com