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Media Release

Basel (Switzerland), April 27, 2017

PIQUR Receives EMA Orphan Drug Designation for PQR309 in Diffuse Large B-Cell Lymphoma

PIQUR Therapeutics AG, a Swiss clinical-stage pharmaceutical company, announced today that the European Medicines Agency (EMA) has granted orphan drug designation to PIQUR's lead compound PQR309 for the treatment of patients with diffuse large B-cell lymphoma (DLBCL).

"The EMA orphan drug designation for PQR309 in DLBCL is another important regulatory milestone, validating the potential therapeutic use of PQR309 in DLBCL," said Claudia Pluess, Senior Regulatory Affairs Manager at PIQUR. Dr. Vladimir Cmiljanovic, CEO of PIQUR, added, "PIQUR will continue to work with physicians and regulatory agencies to further define the clinical development strategy to bring a potential new treatment option to patients suffering from this disease."

DLBCL is an aggressive form of lymphoma, and the most common type of non-Hodgkin lymphoma (NHL), accounting for about 30 percent of all NHL cases [1]. The disease occurs primarily in older individuals, though it can also occur in children and young adults in rare cases. 10 to 15 percent of DLBCL patients exhibit refractory disease and an additional 20 to 25 percent relapse after initial response to therapy [2].

In addition to this orphan drug designation by the EMA in DLBCL, PIQUR has also recently received orphan drug designation from the FDA for PQR309 for the treatment of primary CNS lymphoma (PCNSL).

The EMA orphan drug designation is a status assigned to a medicine intended for use against a rare condition (prevalence of the condition in the European Union must not be more than 5 in 10,000) and allows a pharmaceutical company to benefit from incentives offered by the EU to develop a medicine for the treatment, prevention or diagnosis of a disease that is life threatening or a chronically debilitating rare disease.

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Notes to Editors

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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. 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 several 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). Additional information regarding the PQR309 clinical trials is available on www.clinicaltrials.gov.

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

References

[1] American Society of Clinical Oncology. Lymphoma - Non-Hodgkin: Subtypes (Dec. 2016 revision). http://www.cancer.net/cancer-types/lymphoma-non-hodgkin/subtypes. Accessed April 2017.
[2] Sehn, L. Paramount prognostic factors that guide therapeutic strategies in diffuse large B-cell lymphoma. *Hematology*, December 2012; 1; 402-409.