NEWS RELEASE

qed tx – a new company working on a treatment for achondroplasia

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At the beginning of 2018, **QED Therapeutics** was launched by **Bridge Bio**, as a new biotechnology company, focused on precision medicine for FGFR-driven diseases.

What are FGFRs?

The fibroblast growth factor receptors (FGFRs) regulate important biological processes including cell proliferation (multiplication) and differentiation during development and tissue repair. Over the past decades, numerous pathological conditions and developmental syndromes have emerged as a consequence of deregulation in the FGFRs signaling network. The human fibroblast growth factor receptor (FGFR) family comprises of four family members—FGFR1, FGFR2, FGFR3 and FGFR4. (1)

Achondroplasia is caused by a heterozygous mutation in the fibroblast growth factor receptor-3 gene (FGFR3) on chromosome 4p16.3. (2)

The lead candidate of QED Tx is Infigratinib (BGJ398), that is an orally administered tyrosine kinase inhibitor that blocks FGFR1-FGFR2-FGFR3. It has shown meaningful clinical activity in patients in the oncology/cancer area, specifically with chemotherapy-refractory cholangiocarcinoma FGF2 fusions and metastatic urothelial carcinoma

with FGFR3 genomic alterations. (3)

With the research by **Kombla-Ebri D et al., 2016**, Infigratinib demonstrated also potential in pediatric skeletal dysplasias, including achondroplasia. In this investigation, researchers demonstrated that low doses of infigratinib corrected pathological hallmarks of achondroplasia in mouse models. (4)

In May 2016, it was already published here in Beyond Achondroplasia, a review on NVP-BGJ398: "**New inhibitor of FGFR3 in a mouse model**". NVP-BGJ398 is the same as BGJ398 or Infigratinib. So, to learn more deeply about this compound, read the previous review from 2016. (5)

QED Therapeutics acquired the worldwide rights to infigratinib from Novartis (NVP) for use in all applications and will develop the compound as a treatment for multiple FGFR-driven diseases, including cancers and achondroplasia.

What is precision Medicine?

Is an approach when healthcare is individually tailored on the basis of a person's genes, lifestyle, and environment. Advances in genetics and the growing availability of health data present an opportunity to make precise personalized patient care a clinical reality. Precision medicine is powered by patient data. The health records and genetic codes of patients and healthy volunteers are vital, and help people to influence their own health care and the direction of research. (6)

Stage of clinical studies with Infigratinib

Infigratinib is currently under study in a Phase 2 trial for the treatment of chemotherapy-refractory cholangiocarcinoma (bile duct cancer) with FGFR2 fusions and other activating genomic alterations. (3)

QED is currently evaluating infigratinib in preclinical studies for the treatment of achondroplasia including further efficacy studies and a robust safety program. Pending results from this program, the company intend to begin clinical studies with infigratinib in patients with achondroplasia in 2019.

Sources

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