Deregulation of the Fibroblast Growth Factor Receptor (FGFR) signaling pathway is implicated in various human cancers, including intrahepatic cholangiocarcinoma (CCA), uterine, gastric, breast, and lung cancers. This makes activated FGFRs a promising therapeutic target.

FGFR-FGFR signaling deregulation is associated with FGFR1/2/3/4 genetic aberrations, including aberrant gene expression, amplifications, mutations, translocations, and fusions. In CCA, the optimized combination of FGFR2 vs. 0%, 5.6%, and that of FGFR2/3/4 mutations and aberrations together is estimated at 3%, 6.5%, and 12-17%, similarly.

Dose-dense FGFR inhibitor treatment is well tolerated with manageable adverse effects in patients with cancer. Safety and tolerability data of derazantinib in previous studies and clinical trials showed a low incidence of nail toxicities, stomatitis, hand-foot skin reaction (DILI) were assessed.

- Cochrane Collaboration (type I error 0.0481, power ~0.80 if the true PFS3 rate is p = 0.2) have the lower bound of confidence interval of ORR > 10%.
- 6 months, 12 months, and 18 months: PFS was calculated as Kaplan-Meier survival estimates, analyzed as time-to-event data, and compared between groups using log-rank test under central review for each endpoint.
- Drug-related adverse events (AEs) were defined as follows: all AEs graded using NCI-CTC version 5.0.
- Primary endpoint Cohort 1: Deregulated FGFR2 expression/FGFR2 level.
- Primary endpoint Cohort 2: Progression-free survival (PFS) on 36 months of follow-up.

- The objective response rate and disease control rate was 22.3% / 75.7% in patients with FGFR2 fusions, mutations or amplifications. Median PFS and OS (months) were 7.8 / 17.2 in patients with FGFR2 fusions or amplifications in patients with FGFR2 fusions, mutations or amplifications. Median OS and PFS in patients with FGFR2 fusions, mutations or amplifications. Median OS and PFS in patients with FGFR2 fusions, mutations or amplifications.

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- Treatment: 300 mg derazantinib QD

- RESULTS

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