



## AB014. S3A-3. Precision medicine on the horizon for cholangiocarcinoma

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**Abstract:** Cholangiocarcinoma (CCA) is a molecularly heterogeneous group of tumors, and tumor genotyping efforts have uncovered multiple actionable targets in both intrahepatic and extrahepatic tumors. IDH1 mutations, FGFR2 fusions, and BRAF V600E mutations are among the most promising targets to date in CCA-specific clinical

trials and cohorts, and multiple agents targeting these gene alterations are in development. For patients with IDH1 mutant advanced refractory CCA, a phase III trial of the IDH1 inhibitor AG120 versus placebo recently completed accrual, and the results are anticipated. In FGFR2 fusion positive disease, multiple phase II and III efforts are underway to evaluate the efficacy of selective oral FGFR small molecular inhibitors. In patients with BRAF V600E mutant CCA, the combination of dabrafenib and trametinib has shown promise in a basket trial with a CCA cohort. Additional agents are also under investigation in patients with alterations in HER2, PIK3CA, MET, and CDKN2A and also in patients with mutations in DNA damage repair pathway or chromatin remodeling genes.

**Keywords:** Precision medicine; cholangiocarcinoma; molecular targets

**Cite this abstract as:** Goyal L. Precision medicine on the horizon for cholangiocarcinoma. *HepatoBiliary Surg Nutr* 2019;8(Suppl 1):AB014. doi: 10.21037/hbsn.2019.AB014