

# HCC connect

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# **MEETING SUMMARY**

**AASLD 2016, BOSTON - USA  
NOVEMBER 11<sup>TH</sup> TO 15<sup>TH</sup> 2016**

**DR. SADAHISA OGASAWARA  
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**BIOMARKERS AND MOLECULAR  
PATHOLOGY FOR FUTURE  
TREATMENTS OF HCC**

**FGF19 IMMUNOSTAINING AS  
BIOMARKER FOR TRIAL  
ENRICHMENT TESTING FGFR4  
INHIBITORS IN HCC**

**AGRIN MOEINI ET AL**

**ABSTRACT #1240**

# SUMMARY

- The goal of this study was to evaluate FGF19 as biomarker for trial enrichment with FGFR4 inhibitors and to assess of FGFR4 inhibitor efficacy in HCC patient derived xenograft models
- It was concluded that FGF19 immunostaining accurately identifies patients with FGF19 overexpression, which represent around 25% of HCC population
- These results provide a rationale for trial enrichment in the first in human study testing with selective FGFR4 inhibitors in patients with HCC

# RESULTS

- Overexpression of FGF19: 23% (33/143)
- Focal high level of FGF19: 8% (11/143)
- FGF amplification by FISH assay and NanoString copy number assay: sensitivity = 100%, accuracy = 97%
- FGF19 over over expression by NanoString gene expression assay: accuracy = 83%
- Detection of FGF19 amplification by anti-FGF19 immunostaining: accuracy = 87%
- Detection of FGF19 overexpression by anti FGF19 immunostaining: accuracy = 67%

**AN RNA-BASED SIGNATURE  
ENABLES HIGH SPECIFICITY  
DETECTION OF CIRCULATING  
TUMOR CELLS IN HCC**

**IRUN BHAN ET AL  
ABSTRACT #LB20**

# AN RNA-BASED SIGNATURE ENABLES HIGH SPECIFICITY DETECTION OF CIRCULATING TUMOR CELLS IN HCC

Combining droplet digital PCR with the CTC-iChip yields a high-throughput, highly specific detection mechanism for HCC CTCs

# AN RNA-BASED SIGNATURE ENABLES HIGH SPECIFICITY DETECTION OF CIRCULATING TUMOR CELLS IN HCC

- HCC derived CTCs in 9/16 (56%) untreated HCC patients versus 1/31 (3%) at-risk patients ( $P < 0.0001$ )
  - Positive CTC-scores declined in treated patients: positive scores were found in 9 of 32 (28% patients receiving therapy and only 1/15 (7%) patients who had undergone curative therapies (resection, local ablation, or liver transplantation)
  - Serum AFP and CTC-score together provide superior HCC detection than either single method
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**PHASE II STUDIES WITH  
REFAMETINIB OR REFAMETINIB  
PLUS SORAFENIB IN PATIENTS  
WITH RAS-MUTANT HCC**

**JOSEP M LLOVET ET AL**

**ABSTRACT #1237**

# OBJECTIVE & DESIGN

- Primary objective: the central radiological assessment of objective tumor response (CR + PR) according to mRECIST
- Single arm, open-label, phase II studies in which patients were selected for treatment based on harboring mutations in KRAS/NRAS, determined using cell-free circulating tumor DNA (ctDNA)

# RESULTS

- The presence of RAS mutation of refametinib monotherapy: 6.5% (32/493)
  - The presence of RAS mutation of refametinib + sorafenib: 3.3% (27/815)
  - KRAS/NRAS mutations were detected using confirmatory NGS plasma samples from 12/27 (44.4%) of patients with mutated RAS (refametinib monotherapy: n = 15, refametinib + sorafenib: n = 12); NGS accuracy was 91.7% (11/12).
  - No patients receiving refametinib monotherapy or refametinib + sorafenib combination therapy achieved a CR or PR according to mRECIST
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