Real-world outcomes of gemcitabine-cisplatin plus durvalumab or pembrolizumab in advanced BTC: K-HBP Cancer Alliance study

Changhoon Yoo, Kyunghye Bang, Mooonho Kim, Ju Won Kim, Young-Gyu Park, Chung Ryul Oh, Hyewon Ryu, Ilhwan Kim, Hyehyun Jeong, Hongsik Kim

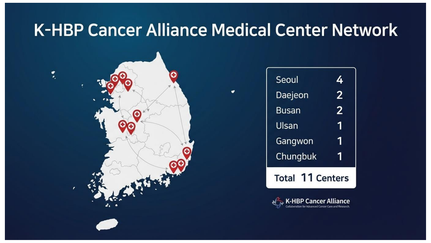
Asan Medical Center, University of Ulsan College of Medicine, Seoul, Konkuk University Medical Center, Konkuk University College of Medicine, Seoul, Gangneung Asan Hospital, Gangneung, Korea University Anam Hospital, Korea University College of Medicine, Seoul, Konyang University Hospital, Daejeon, Chung-Ang University Hospital, Chung-Ang University College of Medicine, Seoul, Chungnam National University Hospital, Chungnam National University College of Medicine, Daejeon, Haeundae Paik Hospital, Inje University College of Medicine, Busan, Chungbuk National University Hospital, Chungbuk National University College of Medicine, Cheongju, Korea.

Background

- Following the positive results of TOPAZ-1 and KEYNOTE-966, gemcitabine plus cisplatin (GemCis) combined with durvalumab or pembrolizumab has been established as a global standard first-line regimen for advanced biliary tract cancer (BTC).
- Because patients treated in routine practice may differ from those enrolled in prospective trials, real-world evaluation of these regimens is warranted.
- In Korea, durvalumab and pembrolizumab have been approved since November 2022.

Methods

- The K-HBP Cancer Alliance comprises 11 tertiary referral hospitals in Korea.



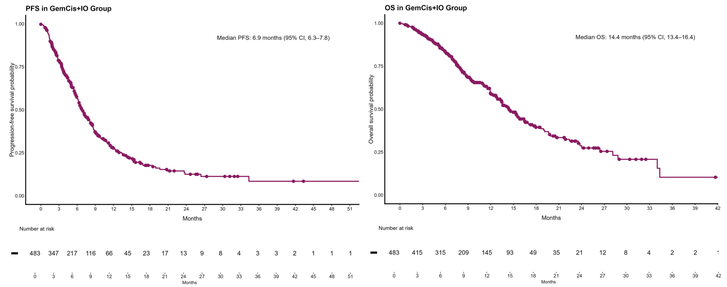
- This multicenter retrospective study included patients who received first-line GemCis plus durvalumab or pembrolizumab for unresectable or metastatic BTC.
- A total of 534 patients who received first-line GemCis plus durvalumab or pembrolizumab between November 2022 and October 2025 were included in this analysis.
- Efficacy outcomes including objective response rates (ORR), progression-free survival (PFS) and overall survival (OS) were analyzed.

Results

- **Patient characteristics**

Characteristic	GemCis+ICI N = 534
Age, median (min–max)	67 (29-89)
<65	221 (41%)
≥65	313 (59%)
Sex	
Male	309 (58%)
Female	225 (42%)
ECOG PS	
0	165 (40%)
1	231 (56%)
≥2	14 (3.4%)
Primary site	
Intrahepatic	211 (40%)
Extrahepatic	212 (40%)
Gallbladder	111 (21%)
Disease status	
Recurrent	173 (32%)
Locally advanced (unresectable)	101 (19%)
Initially metastatic	260 (49%)
HBV	
Negative	494 (93%)
Positive	40 (7%)
HCV	
Negative	530 (99%)
Positive	4 (1%)
Prior curative surgery	173 (32%)
Prior adjuvant chemotherapy	115 (22%)
Site of metastasis	
Lymph node	247 (46%)
Liver	234 (44%)
Lung	77 (14%)
Peritoneum	125 (23%)
Bone	58 (11%)
CA19-9, median (min–max)	115 (1-702,114)

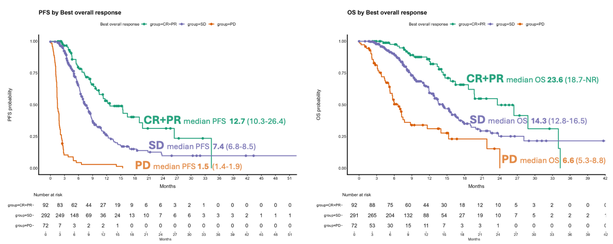
K-M curves for PFS and OS



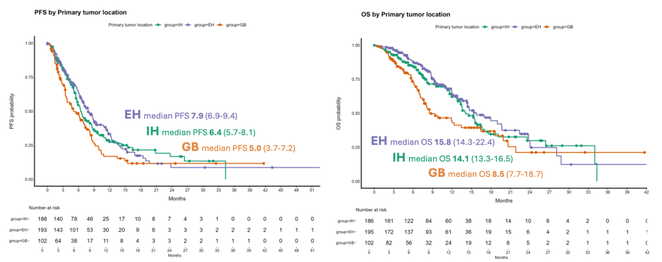
- ***Key efficacy outcomes***

Outcome	GemCis+ICI N = 534
Tumor response	
Response-evaluable population, n (%)	500 (94%)
Objective response rate, n (%) [95% CI]	100 (**20%**) [17–24%]
Disease control rate, n (%) [95% CI]	423 (**85%**) [81–87%]
Best overall response, n (%)	
CR	8 (1%)
PR	92 (17%)
SD	323 (60%)
PD	77 (14%)
NE/NA	34 (6%)
Progression-free survival	
Median PFS, months (95% CI)	**6.6** (6.1–7.2)
6-month PFS rate	55%
12-month PFS rate	26%
24-month PFS rate	13%
Overall survival	
Median OS, months (95% CI)	**14.3** (12.9–15.8)
6-month OS rate	82%
12-month OS rate	58%
24-month OS rate	27%
Follow-up	
Median follow-up, months (95% CI)	**12.0** (10.3–13.5)

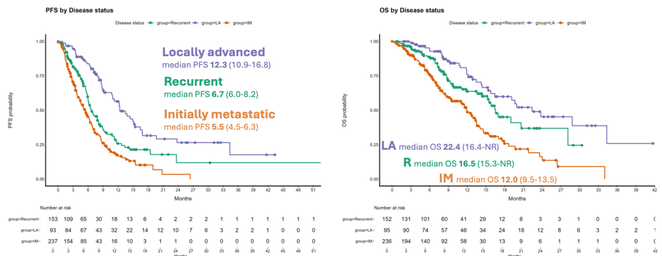
PFS and OS according to the tumor response



PFS and OS according to the primary tumor site



PFS and OS according to the disease setting



Multivariable analysis for OS

Variable	HR (95% CI)	P value
Age, years		
<65	Reference	
≥65	1.13 (0.85-1.50)	0.394
Sex		
Male	Reference	
Female	1.06 (0.80-1.40)	0.667
ECOG PS		
0-1	Reference	
≥2	**2.34** (1.15-4.77)	**0.019**
Primary site		
Intrahepatic	Reference	
Extrahepatic	0.85 (0.62-1.17)	0.319
Gallbladder	1.33 (0.94-1.89)	0.111
Disease status		
Recurrent	Reference	
Locally advanced	**0.61** (0.39-0.97)	**0.035**
Initially metastatic	**1.73** (1.24-2.41)	**0.001**
HBV		
Negative	Reference	
Positive	1.30 (0.81-2.09)	0.281
HCV		
Negative	Reference	
Positive	1.18 (0.16-8.43)	0.870

Variable	HR (95% CI)	P value
Prior curative surgery		
No	Reference	
Yes	**0.07** (0.01-0.56)	**0.012**
Prior adjuvant chemotherapy		
No	Reference	
Yes	1.22 (0.65-2.31)	0.530
LN metastasis		
No	Reference	
Yes	**1.76** (1.33-2.32)	**<0.001**
Liver metastasis		
No	Reference	
Yes	**1.41** (1.07-1.87)	**0.016**
Lung metastasis		
No	Reference	
Yes	1.38 (0.92-2.07)	0.114
Peritoneal metastasis		
No	Reference	
Yes	**1.93** (1.43-2.62)	**<0.001**
Bone metastasis		
No	Reference	
Yes	1.19 (0.72-1.95)	0.504
CA19-9		
<median	Reference	
≥median	**1.51** (1.13-2.03)	**0.006**

Conclusion

- In this large multicenter real-world cohort, GemCis plus durvalumab or pembrolizumab demonstrated efficacy outcomes consistent with prospective trials.
- Tumor response and disease setting were significantly associated with OS.

Corresponding author: Changhoon Yoo, M.D., Ph.D. **E-mail: yooc@amc.seoul.kr**