AB029. S6-3. Clinical trials incorporating translational targets

Takuji Okusaka

Department of Hepatobiliary and Pancreatic Oncology, National Cancer Center Hospital, Tokyo, Japan

Correspondence to: Takuji Okusaka. Department of Hepatobiliary and Pancreatic Oncology, National Cancer Center Hospital, Tokyo, Japan. Email: tokusaka@ncc.go.jp.

Abstract: On the basis of the results of phase III clinical trials in patients with inoperable biliary cancer, gemcitabine and cisplatin combination therapy (GC therapy) has been positioned as a standard global therapy for this cancer. However, patients with biliary cancer still have a rather poor prognosis at present, and the sensitivity of this cancer to existing drug therapies is very low. For these reasons, much has been expected of the development of drugs with new mechanisms of actions, such as molecular-targeted drugs, and at present, clinical trials are under way to determine the efficacy of these agents. Initial trials targeting the epithelial growth factor receptor and angiogenesis pathways have failed to deliver new treatments. Emerging evidence suggests that biliary cancer encompasses subgroups with discrete driver mutations, some of which are targetable with novel therapies. Among the newly discovered molecular alterations, targeting FGFR2 fusions, IDH1/2 mutations and HER2 receptors hold great promise for improving the future management of biliary cancer. Immunotherapy in combination with targeted agents and chemotherapy may improve outcomes. The role of systemic therapies, including targeted therapies for this disease is rapidly evolving.

Keywords: Cholangiocarcinoma; clinical trials; targeted agents; chemotherapy