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Redefining high risk patients with stage II colon cancer

Exiqon A/S (NASDAQ OMX Copenhagen: "EXQ") today announced the publication of data from a population based study of 554 patients with stage II colon cancer, using the company's proprietary miRCURY LNA™ Universal RT microRNA PCR platform to validate the prognostic value of microRNA-21 based on tissue sections from formalin fixed paraffin embedded tumour blocks (FFPE samples). End-points were overall survival (OS) and recurrence free cancer specific survival (RF-CSS).

The study results support microRNA-21 as an independent prognostic biomarker for recurrence free cancer specific survival in a population based cohort of patients with stage II colon cancer, which received surgical intervention. Assessment of microRNA-21 levels was based on qPCR analysis of microRNA-21 in standard FFPE samples, and high microRNA-21 expression was associated with an unfavourable RF-CSS. The cross-validated area under the curve (AUC) for 1- and 5-year RF-CSS were 0.67 and 0.68, respectively. The results appeared in British Journal of Cancer

This study confirms results from a previous study published in British Journal of Cancer in 2012, of the same cohort of patients using the company's proprietary LNA™ based *in situ* hybridization to analyze microRNA-21 levels. The earlier study showed that patients expressing high levels of miR-21 had significantly inferior recurrence-free cancer-specific survival, suggesting that analyses of microRNA-21 should be considered as a potential adjunct in the selection of high risk stage II colon cancer patients.

The new study tested the prognostic value of a qPCR based assessments of microRNA-21 levels in standard FFPE stage II tumour samples in combination with a traditional panel of prognostic biomarkers to create an individual risk index in patients with stage II colon cancer. Individual biomarkers might not be powerful enough to identify clinically relevant risk groups in a cohort of patients where the majority has already been cured by the surgical intervention and has relatively good prognosis. Hence, the aim of the index approach was to strengthen existing markers by combining these traditional biomarkers with the molecular based biomarker microRNA-21 to provide for a clinically more robust stratification.

The study result suggests that introducing a risk index including microRNA-21 for stratifying patients with stage II colon cancer may lead to the identification of a considerably smaller group of high risk patients (23% of the patients at high risk) than the traditional risk parameters (77% of the patients), holding promise to spare a large fraction of patients from needless and harmful adjuvant

chemotherapy. The proposed risk index classifies patients correctly (high or low risk) with a probability of approximately 70%. The cross-validated AUC for the recurrence free cancer specific survival (RF-CSS) index at 1- and 5-year was 0.714 and 0.667, respectively.

The identification of high risk patients is a first step in a possible stratification of patients for adjuvant therapy. The current study does not address whether high risk patients with elevated microRNA-21 expression also will benefit from adjuvant therapy. The possible predictive value of microRNA-21 in identifying high-risk patients who will respond to adjuvant chemotherapy will require further clarification in a clinical context.

With the publication of the current microRNA-21 study, it is clear that microRNA-21 possesses independent prognostic value which in combination with more traditional biomarkers can improve identification of high risk patients. This is a step forward towards better treatment decisions of stage II colon cancer patients who may benefit from being identified as eligible to chemotherapy. Exiqon will proceed with launch of the microRNA-21 test as a RUO (Research Use Only) kit to facilitate further testing and eventually clinical incorporation of microRNA-21 along with other biomarkers to create colorectal cancer recurrence tests,. The launch is planned for later this year.

Additional information

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About Exiqon

Exiqon's products are based on the proprietary LNA™ technology. This technology offers unique advantages for detection of miRNA biomarkers for life science researchers, drug developers and cancer treating physicians working towards personalizing medicine. Exiqon operates in two business areas: Exiqon Life Sciences has established a position for itself as one of the market's leading providers of miRNA research products for miRNA analysis in cells and bodyfluids. Our research products are used by academia, biotech and pharmaceutical companies around the world to make groundbreaking discoveries about the correlation between gene activity and the development of cancer and other diseases. Exiqon Life Sciences is also collaborating with pharmaceutical companies in their effort to target new medicines based on miRNA as biological markers. Exiqon Diagnostics collaborates with pharmaceutical and diagnostic companies to develop novel molecular diagnostic tests for early detection of diseases which can help physicians make treatment decisions. Exiqon is listed on the NASDAQ OMX in Copenhagen. For more information about us, please visit www.exiqon.com.