Diagnostic and therapeutic implications of microRNAs and long noncoding RNA Research

The lecture will discuss recent developments and clinical perspectives noncoding RNA research, with special emphasis on the translation of basic science insights and preclinical research into clinically valuable novel diagnostic tools and therapeutic strategies. Regarding diagnostic value, ncRNA biomarkers (both miRs and lncRNAs) will be critically weighed against current differential diagnostic and prognostic markers. Regarding therapy, ncRNA molecules can be targets as well as tools. The fundamental novelty of these therapies arises from the fact that they exploit tailor-made molecular interactions between endogenous and synthetic ncRNAs. Preclinical animal studies showed high efficacy of anti-miR therapeutics, and of RNA interference (RNAi) strategies employing ncRNAs as tools for silencing of protein-coding genes. An increasing spectrum of endogenous ncRNAs is employed for the development of novel therapeutic ncRNA tools (tRNA and rRNA scaffolds, chimeric tRNA/miR structures). Clinical trials aiming at translation of these strategies into clinical practice have met with variable success so far. The most successful trials addressed precisely defined patient cohorts in whom one pathomechanism was the sole or dominant cause of disease development and progression. Accordingly, future clinical trials are likely to increasingly focus upon patients in whom the high costs and efforts of ncRNA therapeutics development are likely to result in significant clinical success and patient benefit. We discuss selection criteria and suitable clinical outcome parameters, and paradigmatic ncRNA-based strategies at late preclinical stage or in clinical trials are reviewed with regard to clinical translational potential and possible impact upon clinical practice.

Biography

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