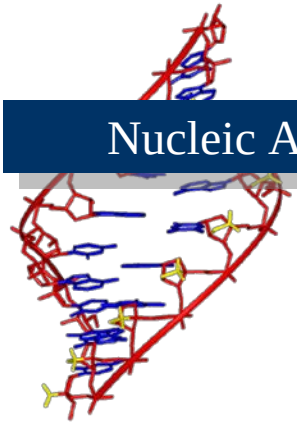
SDU
DEPARTMENT OF PHYSICS, CHEMISTRY AND PHARMACY



Nucleic Acid Center **Seminar** no. 78

Tuesday 30 May 2017
at 11:15 in the FKF Meeting-room

Prof. Leonidas A. Phylactou

The Cyprus Institute of Neurology & Genetics
Provost, Cyprus School of Molecular Medicine,
Nicosia, Cyprus

"Identifying Biomarkers and Novel Therapies for Muscular Dystrophy"

Web: http://www.cing.ac.cy/easyconsole.cfm/id/1/uid/0.873688505955

Abstract:

A major aspect of the research work performed in our laboratory looks into muscle and more specifically into gene therapy and the identification of biomarkers in muscular dystrophy.
Muscular dystrophy is a group of inherited diseases characterized by muscle weakness and wasting. Two major diseases in this category is Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy (DM). Our laboratory is currently working on characterizing the therapeutic efficiency of antisense oligonucleotides (AON) for targeting the genetic defects at the RNA level of both of the above dystrophies. AONs were designed to contain chemically modified nucleotides with Locked Nucleic Acid (LNA) and 2'-OxyMethyl (2'-OMe) incorporations. In parallel, our group has been developing a novel method for delivering more efficiently therapeutic ASOs in muscle. RNA genetic molecules, called aptamers have been identified through an in vitro evolution procedure which exhibit enhanced penetration in muscle in vitro and in vivo. Both of the above programs have shown promising results towards the development of rationale therapies for muscular dystrophy. Finally, our laboratory has been working on the detection of muscle-specific microRNAs (miRNAs) in the blood of patients with DM. More specifically, we have been able to associate the blood levels of certain miRNAs with the progression of the disease. In a follow up study to characterize the ontology of these miRNAs in the blood of patients, we have discovered that they are all encapsulated in circulating exosomes. These results point towards the development of novel biomarkers for the disease and also provide additional information on the pathogenesis of DM.