Special Lecture :

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„Stem Cell Programs in Leukemia and Programming Hematopoietic Stem Cells against Cancer “

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Lecture Hall H, 16:45

Host: Dr. Nico Lachmann **Tel.:** 5266


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Abstract

The role of microRNAs during hematopoiesis, with a particular focus on stem cells and leukemia
The lab disposes of a decade-long experience in microRNA research. We developed microRNA-regulated vectors to finetune transgene expression according to tissue-, lineage- and differentiation stage. Moreover, we developed lentiviral vector platforms for stable miRNA knockdown and overexpression enabling the study of gain- and loss-of-function phenotypes in primary hematopoietic cells. Using this technology, we described the role of miR-223 in murine and human hematopoiesis, miR-155 in tumor-infiltrating myeloid cells and miR-126 in hematopoietic stem cells.
Our current work is focused on studying hematopoietic stem cell microRNAs in normal hematopoiesis and leukemia. In HSC, we aim to understand the molecular mechanism by which miRNAs influence quiescence, self-renewal and commitment and identify actionable targets to modulate HSC functions, e.g. to promote self-renewal and HSC expansion.

Ex vivo gene therapy using hematopoietic stem and progenitor cells
The possibility to genetically manipulate hematopoietic stem and progenitor cells with high efficiency opens up new avenues for the treatment of monogenic disorders affecting hematopoietic tissues. Moreover, HSPC grafts can be engineered to turn them into highly effective therapeutic vehicles with unprecedented new treatment potential. The progeny of HSPC have the capacity to extensively home to tissues including the central nervous system and the tumor microenvironment, capable of delivering a therapeutic payload, e.g. a functional enzyme or an anticancer molecule, into the tissues. At TIGET, we are integrating innovative *ex vivo* gene therapy protocols into our clinical trial pipeline for a variety of monogenic disorders. Moreover, we founded a spin-off company (Genenta.com) developing cancer gene therapy approaches based on the transplantation of HSPC engineered with transcriptionally and post-transcriptionally regulated anti-tumor transgenes, with the ambitious aim to start a phase I/II clinical trial in Multiple Myeloma patients in the next years.