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**KEYWORDS** – Myelofibrosis, Myeloproliferative Neoplasms, patient-derived xenograft

## MAIN FIELDS OF RESEARCH; ABSTRACT

### Development of a patient-derived xenograft model for myelofibrosis

I started my doctoral studies in February 2015 with a focus on developing a patient-derived xenograft (PDX) mouse model for myelofibrosis (MF). Pre-clinical PDX mouse models have emerged as powerful tools for investigating normal and leukemic stem cells (HSCs and LSCs), as well as HSC and LSC heterogeneity. A growing number of models have been developed for aggressive malignancies, such as acute leukemias (AML). However, engraftment of less aggressive malignancies, like MF, is often limited. MF is a HSC disorder characterized by bone marrow fibrosis that has the potential to transform into AML depending on the clonal evolution of MF stem cells. Treatment refractoriness and disease progression remain a major challenge; therefore, there is need for a suitable model. The aim of my project is to use next-generation mice to develop a pre-clinical MF PDX model that will serve as a tool to understand the disease pathogenesis and assess response to novel therapies.

## SPECIAL TECHNIQUES AND EQUIPMENT

Flow Cytometry, FACS cell sorting, xenografts, transgenic mouse models, molecular biology techniques