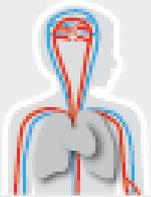


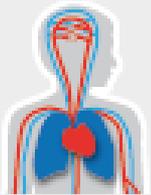
Heart Failure & Arrhythmias



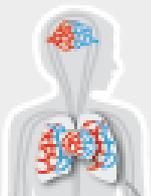
Pulmonary Hypertension
& Thrombosis



Atherosclerosis
& Ischemic Syndromes



Diabetes & Metabolism



Microcirculation

Focus of research group (I)



Amsterdam
Lysosome Center

Name PI: Mirjam Langeveld, MD PhD

Department: Endocrinology and Metabolism, Amsterdam UMC

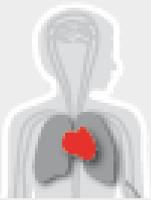
Size of research group

My research group is embedded in SPHINX, the Amsterdam Lysosomal Center. The research is headed by two PI's: Carla Hollak and Mirjam Langeveld. Team members are Simon Korver (PhD student), Sanne van der Veen (PhD student), Mohamed El Sayed (PhD student), Shirley Klein (research nurse), Rika Rhee-Martha (research nurse), Laura van Dussen (epidemiologist) and Mareen Datema (datamanager).

Current mission, vision and aims

My long term research goal for Fabry cardiomyopathy is to:

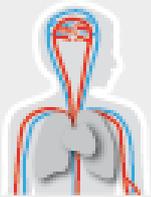
- understand the pathophysiology and identify potential treatment targets
- develop new diagnostic strategies for early disease detection
- define relevant functional outcome parameters of treatment



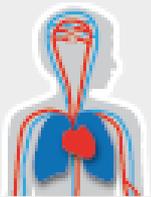
Heart Failure & Arrhythmias



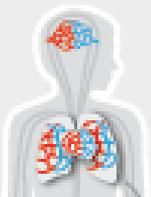
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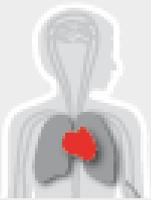
Focus of research group (II)

Current expertise

My research focusses on inherited metabolic diseases in adults, with an emphasis on lysosomal storage disorders (LSD's) and fatty acid oxidation disorders (FAOD). The LSD research is embedded in SPHINX, a collaboration of the departments of Radiology, Internal Medicine and Paediatrics and the Laboratory of Genetic and Metabolic Diseases. With regard to LSD my main focus is Fabry disease, in particular Fabry cardiomyopathy.

Current funding

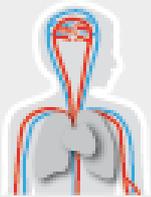
- F20-Study of the Safety and Efficacy of PRX-102 Compared to Agalsidase Beta on Renal Function (BALANCE)
- F30-Extension Study of 1 mg/mL Pegunigalsidase Alfa in Patients With Fabry Disease (BRIDGE)
- International Fabry Genotype Phenotype Study and Open Access Registry Development (ZonMW VIMP round GGG1)
- Stofwisselkracht



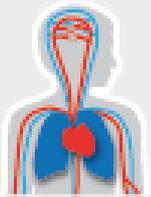
Heart Failure & Arrhythmias



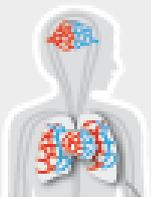
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Microcirculation

Future plans

Short-term plan (1-2 year)

- Study exercise intolerance in Fabry disease and its relationship with early signs of cardiac dysfunction and/or skeletal muscle pathology
- Perform longitudinal study on imaging and electrophysiology characteristics and biomarkers during the development of Fabry cardiomyopathy

Long-term plan (>2 year)

- Develop disease models to study pathophysiology and potential treatment targets for Fabry cardiomyopathy
- Test pharmacological interventions

The necessary infrastructure is available

Collaboration in ACS

- Cardiology (AMC): Matthijs Boekholdt and Pieter Postema
- Physiology (VUMC): Jolanda van der Velden