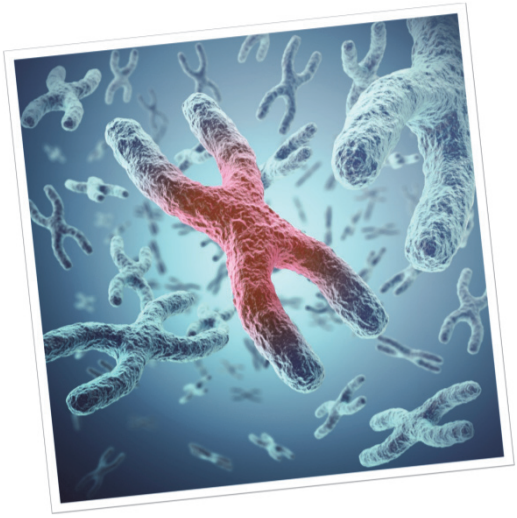
AARHUS
UNIVERSITY
DEPARTMENT OF BIOMEDICINE

# Genetics

# CRISPR/Cas9 Gene Editing

**Rasmus O. Bak**
bak@biomed.au.dk
The Bartholin Building (1242), Room 534
3 postdocs, 1 research assistant

## Background



CRISPR/Cas9 has emerged as a simple, versatile, and efficient tool to make precise changes to the human genome. We are now exploring this technology as a gene therapy to correct mutations in diseases where a defect in a single gene (monogenic) causes devastating disease. Multiple diseases of the blood system are monogenic and can potentially be cured by repairing these mutations in blood stem cells, that due to their stem cell properties can supply the blood system with functional mature blood cells for a life time. An example of such a disease is Severe Combined Immunodeficiency (SCID) where newborn babies lack a functional immune system, and consequently cannot fight off common infections. With CRISPR/Cas9 we hope to be able to correct the responsible mutation and cure patients definitively.

## Projects and techniques

In the lab, we work with a multitude of molecular biology techniques. Examples include:

- Plasmid Cloning
- Virus-based vectors for gene delivery
- Flow Cytometry and cell sorting (FACS)
- Quantitative PCR (qPCR)
- Droplet Digital PCR (ddPCR)
- Blood stem cell isolation
- Colony-forming stem cell assays
- Stem cell transplantation in mice
- Blood production analyses in mice

Student projects may involve a selection of these techniques based on the student's own wishes.

**Relevant publications from the lab:**

1. Bak *et al.* CRISPR/Cas9 genome editing in human hematopoietic stem cells. ***Nature Protocols***, 2018.
2. Bak *et al.* Multiplexed genetic engineering of human hematopoietic stem and progenitor cells using CRISPR/Cas9 and AAV6. ***eLife***, 2017.
3. Bak *et al.* CRISPR-Mediated Integration of Large Gene Cassettes Using AAV Donor Vectors. ***Cell Reports***, 2017.
4. Dever & Bak *et al.* CRISPR/Cas9 β-globin gene targeting in human haematopoietic stem cells. ***Nature***, 2016.
5. Hendel & Bak *et al.* Chemically modified guide RNAs enhance CRISPR-Cas genome editing in human primary cells. ***Nature Biotechnology***, 2015.

***Lab Website Link***