



BSc project

Development of an Antisense Oligonucleotide Therapy for SPNS1

The Dutch Center for RNA Therapeutics (DCRT, rnatherapy.nl) at the Leiden University Medical Center is looking for a Bachelor thesis student or internship student at BSc/MSc level to join us for 3-4 months.

The DCRT is a Dutch-wide non-profit consortium of university medical centers aimed at developing genetic therapies for patients with rare neurological disorders. We are focusing on antisense oligonucleotide therapies that can benefit individual patients or very small groups of patients globally.

We are seeking a student to join us for a project to test antisense oligonucleotides targeting *SPNS1*. Pathogenic variants causing disease in *SPNS1* have only been identified in two families worldwide, affecting a total of three patients currently. The disorder is yet to be described as a new disease entity. For more information on the patient story, please listen to our podcast: <https://www.n1collaborative.org/podcast/episode/21fd73aa/it-takes-a-village>

For this project, we would like to test a set of antisense oligonucleotides and investigate whether we can rescue protein function in a cell culture model.

Techniques:

- Mammalian cell culture: ASO transfections, RNA and protein extraction
- Molecular biology: PCR, RT-qPCR, Western Blot
- Statistical analyses
- Regular project presentations

We are looking for a motivated student that would like to join us in beautiful Leiden for 3-4 months at the earliest convenient time point. Ideally, the student already has experience in wet-lab work, cell culture experience is a plus. We expect the student to be fluent in written and spoken English as communication within our team is in English.

You will join the Department of Human Genetics at the Leiden University Medical Center and become part of a young team of motivated researchers dedicated at developing genetic therapies for rare disease patients.

If you are interested in this project, please reach out to Marlen Lauffer (m.c.lauffer@lumc.nl) with your CV and a short motivation letter describing your interest in this project.