Curriculum vitae W.M.C. Willeke van Roon-Mom

Work experience since graduating

- 2021-present **Professor of translational studies of neurodegenerative diseases**, department of Human Genetics, LUMC, The Netherlands.
- 2020-present **Co-director of the Dutch Center of RNA Therapeutics** , department of Human Genetics, LUMC, The Netherlands.
- 2017-2021 **Associate Professor**, Polyglutamine disease research group, department of Human Genetics, LUMC, The Netherlands.
- 2011-present **Assistant Professor**, Huntington's disease research group, department of Human Genetics, LUMC, The Netherlands.
- 2005-2011 **Post-doctoral researcher fellow**, Huntington's disease research group, department of Human Genetics, LUMC, The Netherlands.
- 2004-2005 **Research Fellow/Post Doctoral research fellow** at the Department of Anatomy with Radiology, University of Auckland, New Zealand
- Lab manager for a research lab. Financial responsibility for all research grants in the lab.

Brief summary of research

Willeke van Roon-Mom is a full professor of Human Genetics, in particular of translational studies of neurodegenerative disorders. She studied Medical Biology at the Rijksuniversiteit in Groningen, and did her PhD in Auckland New Zealand studying Huntingtons disease. After a Post Doc in New Zealand, she returned to the Netherlands to work at the Human Genetics department at the Leiden University Medical Center where she started her own research group. Her work is highly translational in nature, working in close collaboration with clinical departments and industry. Unique patient-driven fund raising initiatives contribute not only financial input, but also patient perspective to research programs in her group. The main topic of her research is autosomal dominant neurodegenerative diseases that have aberrant protein aggregation as a pathological hallmark. She studies molecular disease mechanisms, identifies biomarkers and then uses this knowledge to develop novel therapies with a focus on RNA targeting antisense oligonucleotide therapies. She is the co-founder and co-lead of the Dutch Center for RNA Therapeutics that aims to develop RNA targeting therapies for patients with ultra-rare mutations.
I have been the main supervisor for 12 PhD students and have set on more than 40 PhD review committees both nationally and internationally.

International activities last 5 years

- September 2021 onwards. Member of the Ataxia Global Initiative
- March 2021 onwards. Expert Review Group that will peer review the scientific programmes of the UK Dementia Research Institute (UK DRI)
- 2021. Special Research Topic editor RNA-based Therapeutics: from Bench to Clinic. Frontiers in Genetics
- Editorial Board member of Journal of Therapeutic Advances in Rare Disease. April 2020 onwards.
- Organiser COST Training School: "The guide to antisense therapy development". Porto, Portugal 3-7 Februari 2020
- External independent Ethics Advisor in the H2020CONNECT FET ProAct EU consortium 2019-2024.
- January 2019: Member of scientific PhD defence committee (Georgina Askeland) University of Oslo, Norway
- January 2019: Examiner PhD thesis (Whitney Whitford), University of Auckland, New Zealand.
- 2016-2017: Member of the UK MRC Research Grants Board

International Teaching activities

- LUMC Summer School on Developmental Biology and Regenerative Medicine on October 20, 2021. "Modelling late onset neurodegenerative disorders using induced pluripotent stem cells"
- Invited lecturer at the EuroLife Summer School Biology of Brain Disorders 2019 in Dublin, Ireland
- Workshop organisor on Intellectual Property and medical research at the annual EU Neuromics meeting, March6-9, 2016.
- COST Action BM1207 Lecture in workshop on brain delivery of antisense oligonucleotides Bilbao, Spain, February 3 2016. "Exon skipping approaches for neurodegenerative diseases".

Scholarships and prizes, highlight of last 5 years (>6 million Euro in total)

- 2022-2026: NWO NWA-ORC. CureQ; Total budget € 5.500.000
- 2022-2025: Hersenstichting. SCA7 as a showcase for personalized RNA therapy development in The Netherlands. € 400.000.
- 2021-2022: AFM. Investigating cell-specific pathology in Huntington's disease and spinocerebellar ataxia iPSC-derived brain assembloids before and after treatment with targeted antisense oligonucleotides. € 49.958.
- 2021-2026: ZonMW, PSIDER. Towards a human iPSC neuronal platform for neurodevelopmental disorder therapeutic discovery. € 3.985.975.
- 2021-2022: Cure Rare Disease. Towards treating a SCA3 patient with an RNA therapy. €125.000
- 2021-2024: TKI' Life Sciences and Health. Stepping forward; automated analysis of locomotor behavior in a SCA3 mouse model €424.116.
- 2019-2025: ZonMw Programma Translationeel Onderzoek 2. € 1.499.500,- with additional €500.000 in kind from industry. LUMC budget €475.000 Development and clinical evaluation of a RNA-targeting therapy for Spinocerebellar Ataxia type 1. Project number 446002002.
- 2020-2022: Campagne Team Huntington 547€k. Antisense oligonucleotide disease modifying treatment for Huntington disease Stage 2 and 3.

Publications highlights:

Author on 3 Patents WO2012/018257, WO2015/053624, WO 2017/053781.

Sarah J Tabrizi, *et al.*. Potential disease modifying therapies for Huntington's disease – lessons learned and future opportunities. Accepted in The Lancet Neurology

Hannah S. Bakels, *et al.*. Juvenile onset Huntington Disease Pathophysiology and Neurodevelopment: a Review. Accepted in Movement Disorders September 2021

Matthis Synofzik, *et al.*. Preparing n-of-1 ASO treatments for rare neurological diseases in Europe: genetic, regulatory and ethical perspectives. Nucleic Acid Therapeutics September 2021 doi: 10.1089/nat.2021.0039.

Suzan M Hammond, *et al.*. Delivery of Oligonucleotide-Based Therapeutics: Challenges and Opportunities. EMBO Mol Med. 2021 Apr 9;13(4)

Marjolein Bulk; *et al.*. Pathological characterization of T2*-weighted MRI contrast in the striatum of Huntington's disease patients. Neuroimage Clin. 2020;28:102498.

Linda M. van der Graaf, *et al.*. Generation of 5 induced pluripotent stem cell lines, LUMCi007-A and B and LUMCi008-A, B and C, from 2 patients with Huntington

Toonen Lodewijk J.A., *et al.*. Intracerebroventricular Administration of a 2'-O-Methyl Phosphorothioate antisense oligonucleotide results in activation of the innate immune system in mouse brain. Nucleic Acids Therapeutics 2018 Apr;28(2):63-73

Willeke M.C. van Roon-Mom, *et al.*. Dose dependent lowering of mutant huntingtin using antisense oligonucleotides in Huntington disease patients. Nucleic Acid Ther. 2018 Apr;28(2):59-62

Menno H. Schut, *et al.*. Effect of Post-Mortem Delay on N-terminal Huntingtin Protein Fragments in Human Control and Huntington Disease Brain Lysates. PlosOne May 2017, Jun 1;12(6):e0178556.