Prof. Dr. Annemieke Aartsma-Rus is a professor of Translational Genetics at the Department of Human Genetics of the Leiden University Medical Center (LUMC, the Netherlands. In 2020 she co-founded the Dutch Center for RNA Therapeutics (DCRT), a non-for-profit academic collaboration aiming to develop clinical treatment with exon skipping therapies for eligible patients with unique mutations. In 2022 she became a board member of the N-of-1 collaborative (N1C), a global umbrella organization aiming to facilitate development of antisense oligonucleotide therapies for patients with very rare, eligible mutations.

Her work currently focuses on developing antisense-mediated exon skipping as a therapy for Duchenne muscular dystrophy and rare brain diseases. This involves work in cell and animal models to improve efficiency of exon skipping, studies in muscle pathology, studying the basics of pre-mRNA splicing and transcript processing and the generation and detailed analysis of mouse models. Finally, she aims to bridge the gap between stakeholders (patients, academics, regulators and industry) involved in drug development for rare diseases and to develop exon skipping therapies for patients with unique mutations.

She has published over 250 peer-reviewed papers, 11 book chapters and 15 patents. In 2011 she received the Duchenne Award from the Dutch Duchenne Parent Project in recognition of her dedication to the Duchenne field. In 2020 she received the Black Pearl Science Award from Eurordis for her work in educating patients. In 2021 she received the Ammodo Science Award for her contribution to developing exon skipping therapies for Duchenne, the outstanding achievement award from the Dutch Society of Gene and Cell Therapy for her work and the Rosalind Franklin in Science award for her work for the journal Nucleic Acid Therapeutics. From 2015-2023 she was the most influential scientist in Duchenne muscular dystrophy in the past 10 years 8 times in a row (https://expertscape.com/ex/muscular+dystrophy%2C+duchenne).

She is chair of the TREAT-NMD Advisory Committee for Therapeutics (TACT), Chair of the International Rare Disease Research Consortium (IRDiRC) taskforce on N=1 therapies and was President of the Oligonucleotide Therapeutics Society (2019-2021).

She has successfully applied for numerous grant applications, including a VIDI award (€800,000) from the Dutch government (ZonMw) in 2009 and collaborative grants in 2012 (Rare Disease Program, €3,000,000) and 2021 (PSIDER, €4,000,000). She is/was involved in multiple EU projects, e.g. TREAT-NMD (FP6), Bio-NMD (FP7), NeurOmics (FP7), BIND (Horizon2020) and COST Actions BM1208 and CA17103.

She has been instrumental in setting up training courses, e.g. TREAT-NMD Duchenne masterclasses, the TREAT-NMD/EURO-NMD translational summer school and a COST training school on oligonucleotide therapy development (2020**).** She is co-editor in chief of Nucleic Acid Therapeutics, and serves on multiple editorial boards, e.g. Journal of Neuromuscular Diseases (associate editor), Molecular Therapy and Therapeutic Advances in Rare Disease.