HEINRICH WIELAND PRIZE

SYMPOSIUM SPEAKER 2025 | PROFILE

Professor Dr Annemieke Aartsma-Rus
Leiden University Medical Center (LUMC), The Netherlands

Annemieke Aartsma-Rus is a global leader in RNA-based therapies for rare genetic diseases. She pioneered the antisense-mediated exon skipping approach for Duchenne muscular dystrophy (DMD). This approach uses antisense oligonucleotides (ASOs) to skip exons in the dystrophin transcript during pre-mRNA splicing. This restores the genetic code and allows muscle cells to produce a shortened but functional dystrophin protein. Annemieke Aartsma-Rus played a key role in developing this mutation-specific therapy, proving its effectiveness in cell and animal models, and advancing it into clinical trials. Currently, the U.S. Food and Drug Administration (FDA) has approved 4 ASOs for DMD, 3 of which based on the work of Annemieke Aartsma-Rus. Her current research focuses on optimizing ASO therapy for DMD and developing personalized therapies for neurological diseases caused by rare genetic mutations.

Annemieke Aartsma-Rus studied Biomedical Sciences at Leiden University Medical Center (LUMC), The Netherlands, and received her PhD in 2005. She became assistant professor in 2007 and Professor of Translational Genetics at LUMC in 2015. Since 2013, she has been a visiting professor at Newcastle University, UK. In 2020, she co-founded the Dutch Center for RNA Therapeutics and, since 2025 she leads the scientific advisory board of the N-of-1 collaborative to promote ASO therapies for rare mutations. She has been recognized as the most influential scientist in the DMD field for eleven consecutive years and has received multiple awards, including the Duchenne Award and the Black Pearl Science Award.

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