



Curriculum Vitae

Personal information **Annemieke Aartsma-Rus**

Work experience

Present positions

Since 2015 Professor (Hoogleraar) of Translational Genetics at the Department of Human Genetics, Leiden University Medical Center (LUMC)

Since 2013 Visiting professor at the Institute of Genetic Medicine, Newcastle University, Newcastle, UK

Previous positions

2010 - 2015 Associate professor (UHD) at the Dept of Human Genetics, LUMC

2007 –2010 Assistant Professor, Dept of Human Genetics, LUMC

2007. Postdoctoral fellow, Dept. of Human Genetics, LUMC

2004. PhD student, Dept. of Human Genetics, LUMC

2000 (Jan-June) Research Technician, Biomedical Primate Research Center, Rijswijk, the Netherlands

Education and training

2000 MSc Biomedical Sciences, Leiden University (The Netherlands)

2005 PhD: "Development of an antisense-mediated exon skipping therapy for Duchenne muscular dystrophy. Making sense out of nonsense", supervised by prof dr G.J.B. van Ommen, head of the Department of Human genetics, Leiden University Medical Centre.

2015 University Teaching Qualification (Basis Kwalificatie Onderwijs, an accreditation standard for teaching from Dutch Universities)

Additional information

Publications

H-Index: 57 (ISI Web of Science, cited 12,152 times)

H-Index: 68 (Google Scholar; cited 19,526 times); I10index: 208

H-Index: 60 (Researchgate: research interest score: 6,687.5; citations 14,337, reads 58,521)

Number of first author publications: 49

Number of last author publications: 57

Scientific journal publications

1. Johansson C, Hunt H, Signorelli M, Edfors F, Hober A, Svensson AS, Tegel H, Forstström B, **Aartsma-Rus A**, Niks E,

- Spitali P, Uhlén M, Szgyarto CA.: Orthogonal proteomics methods warrant the development of Duchenne muscular dystrophy biomarkers. *Clin Proteomics*. 2023 Jun 12;20(1):23. doi: 10.1186/s12014-023-09412-1.
2. Cameron D, Abbassi-Daloui T, Heezen LGM, van de Velde NM, Koeks Z, Veeger TTT, Hooijmans MT, El Abdellaoui S, van Duinen SG, Verschuuren JJGM, van Putten M, **Aartsma-Rus A**, Raz V, Spitali P, Niks EH, Kan HE: Diffusion-tensor magnetic resonance imaging captures increased skeletal muscle fibre diameters in Becker muscular dystrophy. *J Cachexia Sarcopenia Muscle*. 2023 May 1. doi: 10.1002/jcsm.13242.
 3. van Deutekom J, Beekman C, Bijl S, Bosgra S, van den Eijnde R, Franken D, Groenendaal B, Harquouli B, Janson A, Koevoets P, Mulder M, Muilwijk D, Peterburgska G, Querido B, Testerink J, Verheul R, de Visser P, Weij R, **Aartsma-Rus A**, Puoliväli J, Bragge T, O'Neill C, Datson NA. Next Generation Exon 51 Skipping Antisense Oligonucleotides for Duchenne Muscular Dystrophy. *Nucleic Acid Ther*. 2023 Apr 10. doi: 10.1089/nat.2022.0063
 4. Signorelli M, Tsonaka R, **Aartsma-Rus A**, Spitali P.: Multiomic characterization of disease progression in mice lacking dystrophin. *PLoS One*. 2023 Mar 31;18(3):e0283869. doi: 10.1371/journal.pone.0283869
 5. **Aartsma-Rus AM**: The future of exon skipping for Duchenne muscular dystrophy. *Hum Gene Ther*. 2023 Mar 16. doi: 10.1089/hum.2023.026
 6. **Aartsma-Rus A**, De Waele L, Houwen-Opstal S, Kirschner J, Krom YD, Mercuri E, Niks EH, Straub V, van Duyvenvoorde HA, Vroom E.: The Dilemma of Choice for Duchenne Patients Eligible for Exon 51 Skipping The European Experience. *J Neuromuscul Dis*. 2023 Mar 6. doi: 10.3233/JND-221648
 7. Q&A with **Annemieke Aartstma-Rus** on Individualized ASO therapy for rare diseases. *Commun Med (Lond)*. 2023 Feb 28;3(1):27. doi: 10.1038/s43856-023-00255-3.
 8. Muntoni F, Signorovitch J, Sajeev G, Lane H, Jenkins M, Dieye I, Ward SJ, McDonald C, Goemans N, Niks EH, Wong B, Servais L, Straub V, Guglieri M, de Groot IJM, Chesshyre M, Tian C, Manzur AY, Mercuri E, **Aartsma-Rus A.**: DMD Genotypes and Motor Function in Duchenne Muscular Dystrophy: A Multi-institution Meta-analysis With Implications for Clinical Trials. *Neurology*. 2023 Feb 1:10.1212/WNL.0000000000201626. doi: 10.1212/WNL.0000000000201626.
 9. **Aartsma-Rus A**, van Roon-Mom W, Lauffer M, Siezen C, Duijndam B, Coenen-de Roo T, Schüle R, Synofzik M, Graessner H.: Development of tailored splice switching oligonucleotides for progressive brain disorders in Europe: development, regulation and implementation considerations. *RNA*. 2023 Jan 20:rna.079540.122. doi: 10.1261/rna.079540.122.
 10. Filonova G, **Aartsma-Rus A.**: Next steps for the optimization of exon therapy for Duchenne muscular dystrophy. *Expert Opin Biol Ther*. 2023 Jan 21:1-11. doi: 10.1080/14712598.2023.2169070.
 11. **Aartsma-Rus A.**: Good news for the mdx mouse community: Improved dystrophin restoration after skipping mouse dystrophin exon 23. *Mol Ther Nucleic Acids*. 2022 Nov 8;30:355-356. doi: 10.1016/j.omtn.2022.10.009.
 12. **Aartsma-Rus A**, van Putten M, Mantuano P, De Luca A.: On the use of D2.B10-Dmdmdx/J (D2.mdx) Versus C57BL/10ScSn-Dmdmdx/J (mdx) Mouse Models for Preclinical Studies on Duchenne Muscular Dystrophy: A Cautionary Note from Members of the TREAT-NMD Advisory Committee on Therapeutics. *J Neuromuscul Dis*. 2022 Oct 31. doi: 10.3233/JND-221547.
 13. Engelbeen S, Pasteuning-Vuhman S, Boertje-van der Meulen J, Parmar R, Charisse K, Sepp-Lorenzino L, Manoharan M, **Aartsma-Rus A**, van Putten M: Efficient Downregulation of Alk4 in Skeletal Muscle After Systemic Treatment with Conjugated siRNAs in a Mouse Model for Duchenne Muscular Dystrophy. *Nucleic Acid Ther*. 2022 Oct 20. doi: 10.1089/nat.2022.0021.
 14. Chey YCJ, Arudkumar J, **Aartsma-Rus A**, Adikusuma F, Thomas PQ. :CRISPR applications for Duchenne muscular dystrophy: From animal models to potential therapies. *WIREs Mech Dis*. 2022 Jul 31:e1580. doi: 10.1002/wsbm.1580
 15. Alqallaf A, Engelbeen S, Palo A, Cutrupi F, Tanganyika-de Winter C, Plomp J, Vaiyapuri S, **Aartsma-Rus A**, Patel K, van Putten M. The therapeutic potential of soluble activin type IIB receptor treatment in a limb girdle muscular dystrophy type 2D mouse model. *Neuromuscul Disord*. 2022 May;32(5):419-435. doi: 10.1016/j.nmd.2022.03.002.
 16. Duan D, Flanigan KM, **Aartsma-Rus A**. Letter by Duan et al Regarding Article, "Therapeutic Exon Skipping Through a CRISPR-Guided Cytidine Deaminase Rescues Dystrophic Cardiomyopathy In Vivo". *Circulation*. 2022 May 3;145(18):e872-e873. doi: 10.1161/CIRCULATIONAHA.121.058714.
 17. **Aartsma-Rus A**, Dooms, M, Le Cam Y, OD Expert Group and Copenhagen Economics: Orphan Medicine Incentives: How to Address the Unmet Needs of Rare Disease Patients by Optimizing the European Orphan Medicinal Product Landscape

- Guiding Principles and Policy Proposals by the European Expert Group for Orphan Drug Incentives (OD Expert Group). *Frontiers in Pharmacology*. 2021 Dec 16. Doi 10.3389/fphar.2021.744532.
18. Evéquo D, Verhaart IEC, van de Vijver D, Renner W, **Aartsma-Rus A**, Leumann CJ: 7',5'-alpha-bicyclo-DNA: new chemistry for oligonucleotide exon splicing modulation therapy. *Nucleic Acids Res*. 2021 Dec 2;49(21):12089-12105. doi: 10.1093/nar/gkab1097.
 19. **Aartsma-Rus A**, Vroom E, O'Reilly D: The Role of Patient Involvement When Developing Therapies. *Nucleic Acid Ther*. 2021 Oct 1. doi: 10.1089/nat.2021.0048.
 20. Synofzik M, van Roon-Mom WMC, Marckmann G, van Duyvenvoorde HA, Graessner H, Schüle R, **Aartsma-Rus A**: Preparing n-of-1 Antisense Oligonucleotide Treatments for Rare Neurological Diseases in Europe: Genetic, Regulatory, and Ethical Perspectives. *Nucleic Acid Ther*. 2021 Sep 29. doi: 10.1089/nat.2021.0039. Online ahead of print.
 21. Alghamdi F, Al-Tawari A, Alrohaif H, Alshuaibi W, Mansour H, **Aartsma-Rus A** and Mégarbané A: Case Report: The Genetic Diagnosis of Duchenne Muscular Dystrophy in the Middle East. *Front. Pediatr.*, 13 September 2021. <https://doi.org/10.3389/fped.2021.716424>
 22. **Aartsma-Rus A**: 'N of 1' therapies need a better model. *Nat Med*. 2021 Jun;27(6):939. doi: 10.1038/s41591-021-01380-z.
 23. Tsonaka R, Seyer A, **Aartsma-Rus A**, Spitali P.: Plasma lipidomic analysis shows a disease progression signature in mdx mice. *Sci Rep*. 2021 Jun 21;11(1):12993. doi: 10.1038/s41598-021-92406-6.
 24. Marchal GA, van Putten M, Verkerk AO, Casini S, Putker K, van Amersfoorth SCM, **Aartsma-Rus A**, Lodder EM, Remme CA.: Low human dystrophin levels prevent cardiac electrophysiological and structural remodelling in a Duchenne mouse model. *Sci Rep*. 2021 May 7;11(1):9779. doi: 10.1038/s41598-021-89208-1.
 25. Hammond SM, **Aartsma-Rus A**, Alves S, Borgos SE, Buijsen RAM, Collin RWJ, Covello G, Denti MA, Desviat LR, Echevarría L, Foged C, Gaina G, Garanto A, Goyenvalle AT, Guzowska M, Holodnuka I, Jones DR, Krause S, Lehto T, Montolio M, Van Roon-Mom W, Arechavala-Gomez V.: Delivery of oligonucleotide-based therapeutics: challenges and opportunities. *EMBO Mol Med*. 2021 Apr 9;13(4):e13243
 26. Signorelli M, Ebrahimpoor M, Veth O, Hettne K, Verwey N, García-Rodríguez R, Tanganyika-deWinter CL, Lopez Hernandez LB, Escobar Cedillo R, Gómez Díaz B, Magnusson OT, Mei H, Tsonaka R, **Aartsma-Rus A**, Spitali P.: Peripheral blood transcriptome profiling enables monitoring disease progression in dystrophic mice and patients. *EMBO Mol Med*. 2021 Apr 9;13(4)
 27. Gravesteijn G, Hack RJ, van Opstal AM, van Eijnden BJ, Middelkoop HAM, Rodriguez Gironde MDM, **Aartsma-Rus A**, van de Grond J, Rutten JW, Lesnik Oberstein SAJ: Eighteen-Year Disease Progression and Survival in CADASIL; *J Stroke* 2021 Jan;23(1):132-134.
 28. Duan D, Goemans N, Takeda S, Mercuri E, **Aartsma-Rus A**: Duchenne muscular dystrophy. *Nat Rev Dis Primers*. 2021 Feb 18;7(1):13.
 29. Alvelos MI, Brüggemann M, Sutandy FR, Juan-Mateu J, Colli ML, Busch A, Lopes M, Castela Â, Aartsma-Rus A, König J, Zarnack K, Eizirik DL. : The RNA-binding profile of the splicing factor SRSF6 in immortalized human pancreatic β -cells. *Life Sci Alliance*. 2020 Dec 29;4(3):e202000825.
 30. Yavas A, Weij R, van Putten M, Kourkouta E, Beekman C, Puoliväli J, Bragge T, Ahtoniemi T, Knijnenburg J, Hoogenboom ME, Ariyurek Y, Aartsma-Rus A, van Deutekom J, Datson N.: Detailed genetic and functional analysis of the hDMDdel52/mdx mouse model. *PLoS One*. 2020 Dec 23;15(12):e0244215.
 31. Hiller M, Geissler M, Janssen G, van Veelen P, **Aartsma-Rus A**, Spitali P.: The mRNA Binding Proteome of Proliferating and Differentiated Muscle Cells. *Genomics Proteomics Bioinformatics*. 2020 Dec 15:S1672-0229(20)30134-0.
 32. Schneider AE, **Aartsma-Rus A**: Developments in reading frame restoring therapy approaches for Duchenne muscular dystrophy. *Expert Opin Biol Ther*. 2020
 33. Verhaart IEC, Cappellari O, Tanganyika-de Winter CL, Plomp JJ, Nnorom S, Wells KE, Hildyard JCW, Bull D, **Aartsma-Rus A**, Wells DJ: Simvastatin Treatment Does Not Ameliorate Muscle Pathophysiology in a Mouse Model for Duchenne Muscular Dystrophy. *J Neuromuscul Dis*. 2020 3.
 34. Lyu P, Yoo KW, Yadav MK, Atala A, **Aartsma-Rus A**, Putten MV, Duan D, Lu B: Sensitive and reliable evaluation of single-cut sgRNAs to restore dystrophin by a GFP-reporter assay. *PLoS One*. 2020 Sep 24;15(9):e0239468.
 35. Passarelli C, Selvatici R, Carrieri A, Di Raimo FR, Falzarano MS, Fortunato F, Rossi R, Straub V, Bushby K, Reza M,

- Zharaieva I, D'Amico A, Bertini E, Merlini L, Sabatelli P, Borgiani P, Novelli G, Messina S, Pane M, Mercuri E, Claustres M, Tuffery-Giraud S, **Aartsma-Rus A**, Spitali P, T'Hoën PAC, Lochmüller H, Strandberg K, Al-Khalili C, Kotelnikova E, Lebowitz M, Schwartz E, Muntoni F, Scapoli C, Ferlini A: Tumor Necrosis Factor Receptor SF10A (TNFRSF10A) SNPs Correlate With Corticosteroid Response in Duchenne Muscular Dystrophy. *Front Genet.* 2020 Jul 3;11:605.
36. García-Rodríguez R, Hiller M, Jiménez-Gracia L, van der Pal Z, Balog J, Adamzek K, **Aartsma-Rus A**, Spitali P: Premature Termination Codons in the DMD Gene Cause Reduced Local mRNA Synthesis. *Proc Natl Acad Sci U S A* 2020 Jul 2 ebur
37. Kuijper EC, Bergsma AJ, Pijnappel WWMP, **Aartsma-Rus A**: Opportunities and challenges for antisense oligonucleotide therapies.. *J Inherit Metab Dis.* 2020 May 11.
38. Strandberg K, Ayoglu B, Roos A, Reza M, Niks E, Signorelli M, Fasterius E, Pontén F, Lochmüller H, Domingos J, Ala P, Muntoni F, **Aartsma-Rus A**, Spitali P, Nilsson P, Szegarty CA: Blood-derived biomarkers correlate with clinical progression in Duchenne muscular dystrophy. *J Neuromuscul Dis.* 2020;7(3):231-246.
39. **Annemieke Aartsma-Rus**. *Lancet Neurol.* 2020 May;19(5):388.
40. Hechtelt Jonker A, Hivert V, Gabaldo M, Batista L, O'Connor D, **Aartsma-Rus A**, Day S, Sakushima K, Ardigo D. Boosting delivery of rare disease therapies: the IRDiRC Orphan Drug Development Guidebook. *.Nat Rev Drug Discov.* 2020 Apr 20.
41. Krishnan VS, **Aartsma-Rus A**, Overzier M, Lutz C, Bogdanik L, Grounds MD. Implications of increased S100β and Tau5 proteins in dystrophic nerves of two mdx mouse models for Duchenne muscular dystrophy. *Mol Cell Neurosci.* 2020 Mar 30:103484.
42. van Putten M, Hmeljak J, **Aartsma-Rus A**, Dowling JJ. Moving neuromuscular disorders research forward: from novel models to clinical studies. *Dis Model Mech.* 2020 Feb 25;13(2).
43. Willmann R, Lee J, Turner C, Nagaraju K, **Aartsma-Rus A**, Wells DJ, Wagner KR, Csimma C, Straub V, Grounds MD, De Luca A. Improving translatability of preclinical studies for neuromuscular disorders: lessons from the TREAT-NMD Advisory Committee for Therapeutics (TACT). *Dis Model Mech.* 2020 Feb 7;13(2).
44. **Aartsma-Rus A**, Corey DR. The 10th Oligonucleotide Therapy Approved: Golodirsen for Duchenne Muscular Dystrophy. *Nucleic Acid Ther.* 2020
45. Tsonaka R, Signorelli M, Sabir E, Seyer A, Hettne K, **Aartsma-Rus A**, Spitali P. Longitudinal metabolomic analysis of plasma enables modeling disease progression in Duchenne muscular dystrophy mouse models. *Hum Mol Genet.* 2020 Jan 10
46. Hellebrekers DMJ, Doorenweerd N, Sweere DJJ, van Kuijk SMJ, **Aartsma-Rus AM**, Klinkenberg S, Vles JSH, Hendriksen JGM. Longitudinal follow-up of verbal span and processing speed in Duchenne muscular dystrophy. *Eur J Paediatr Neurol.* 2020 Jan 7
47. Gravesteijn G, Dauwerse JG, Overzier M, Brouwer G, Hegeman I, Mulder AA, Baas F, Kruit MC, Terwindt GM, van Duinen SG, Jost CR, **Aartsma-Rus A**, Lesnik Oberstein SAJ, Rutten JW. Naturally occurring NOTCH3 exon skipping attenuates NOTCH3 protein aggregation and disease severity in CADASIL patients. *Hum Mol Genet.* 2020 Jan 21.
48. Spitali P, Zaharieva I, Bohringer S, Hiller M, Chaouch A, Roos A, Scotton C, Claustres M, Bello L, McDonald CM, Hoffman EP; CINRG Investigators, Koeks Z, Eka Suchiman H, Cirak S, Scoto M, Reza M, 't Hoen PAC, Niks EH, Tuffery-Giraud S, Lochmüller H, Ferlini A, Muntoni F, **Aartsma-Rus A**. TCTEX1D1 is a genetic modifier of disease progression in Duchenne muscular dystrophy. *Eur J Hum Genet.* 2020 Jan 2.
49. Krooss S, Werwitzke S, Kopp J, Rovai A, Varnholt D, Wachs AS, Goyenvalle A, **Aartsma-Rus A**, Ott M, Tiede A, Langemeier J, Bohne J. Pathological mechanism and antisense oligonucleotide-mediated rescue of a non-coding variant suppressing factor 9 RNA biogenesis leading to hemophilia B. *PLoS Genet.* 2020 Apr 8;16(4):e1008690.
50. Signorelli M, Ayoglu B, Johansson C, Lochmüller H, Straub V, Muntoni F, Niks E, Tsonaka R, Persson A, **Aartsma-Rus A**, Nilsson P, Al-Khalili Szegarty C, Spitali P. Longitudinal serum biomarker screening identifies malate dehydrogenase as candidate prognostic biomarker for Duchenne muscular dystrophy. *J Cachexia Sarcopenia Muscle.* 2019 Dec 27.
51. Verwey N, Gazzoli I, Krause S, Mamchaoui K, Mouly V, **Aartsma-Rus A**. Antisense-Mediated Skipping of Dysferlin Exons in Control and Dysferlinopathy Patient-Derived Cells. *Nucleic Acid Ther.* 2019 Dec 23.
52. van Westering TLE, Lomonosova Y, Coenen-Stass AML, Betts CA, Bhomra A, Hulsker M, Clark LE, McClorey G, **Aartsma-Rus A**, van Putten M, Wood MJA, Roberts TC. Uniform sarcolemmal dystrophin expression is required to prevent extracellular microRNA release and improve dystrophic pathology. *J Cachexia Sarcopenia Muscle.* 2019 Dec 17.

53. Aartsma-Rus A, Watts JK. The Munich Meeting: Medical Maturation, More Mechanisms, and Milasen. *Nucleic Acid Ther.* 2019 Dec;29(6):302-304.
54. Gravesteijn G, Munting LP, Overzier M, Mulder AA, Hegeman I, Derieppe M, Koster AJ, van Duinen SG, Meijer OC, Aartsma-Rus A, van der Weerd L, Jost CR, van den Maagdenberg AMJM, Rutten JW, Lesnik Oberstein SAJ. Progression and Classification of Granular Osmiophilic Material (GOM) Deposits in Functionally Characterized Human NOTCH3 Transgenic Mice. *Transl Stroke Res.* 2019 Oct 30
55. Southall NT, Natarajan M, Lau LPL, Jonker AH, Deprez B, Williams T, Hunter L, Rademaker CM, Hivert V, Ardigò D; IRDiRC Data Mining and Repurposing Task Force. The use or generation of biomedical data and existing medicines to discover and establish new treatments for patients with rare diseases - recommendations of the IRDiRC Data Mining and Repurposing Task Force. *Orphanet J Rare Dis.* 2019 Oct 15;14(1):225.
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57. Patel AM, Wierda K, Thorrez L, van Putten M, De Smedt J, Ribeiro L, Tricot T, Gajjar M, Duelen R, Van Damme P, De Waele L, Goemans N, Tanganyika-de Winter C, Costamagna D, **Aartsma-Rus A**, van Duyvenvoorde H, Sampaolesi M, Buyse GM, Verfaillie CM. Dystrophin deficiency leads to dysfunctional glutamate clearance in iPSC derived astrocytes. *Transl Psychiatry.* 2019 Aug 21;9(1):200
58. **Aartsma-Rus A**, den Dunnen JT. Phenotype predictions for exon deletions/duplications: A user guide for professionals and clinicians using Becker and Duchenne muscular dystrophy as examples. *Hum Mutat.* 2019 Jul 29
59. Verhaart IEC and **Aartsma-Rus A**. Therapeutic developments for Duchenne muscular dystrophy. *Nat Rev Neurol.* 2019 May 30. doi: 10.1038/s41582-019-0203-3.
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61. **Aartsma-Rus A**, Krieg AM. Advancing Nucleic Acid Therapeutics by Setting Uniform Standards for Experimental Controls. *Nucleic Acid Ther.* 2019 Apr 9.
62. Van Putten M, Putker K, Overzier M, Adamzek WA, Pasteuning-Vuhman S, Plomp JJ, **Aartsma-Rus A**. Natural disease history of the D2 -mdx mouse model for Duchenne muscular dystrophy. *FASEB J.* 2019 Apr 1
63. Olie CS, Riaz M, Konietzny R, Charles PD, Pinto-Fernandez A, Kiełbasa SM, **Aartsma-Rus A**, Goeman JJ, Kessler BM, Raz V. Deacetylation Inhibition Reverses PABPN1-Dependent Muscle Wasting. *iScience.* 2019 Jan 22;12:318-332.
64. van Putten M, Tanganyika-de Winter C, Bosgra S and **Aartsma-Rus A**. Nonclinical Exon Skipping Studies with 2'-O-Methyl Phosphorothioate Antisense Oligonucleotides in mdx and mdx-utrn^{-/-} Mice Inspired by Clinical Trial Results. *Nucleic Acid Ther.* 2019 Jan 23.
65. Gravesteijn G, Rutten JW, Verberk IMW, Böhringer S, Liem MK, van der Grond J, **Aartsma-Rus A**, Teunissen CE, Lesnik Oberstein SAJ. Serum Neurofilament light correlates with CADASIL disease severity and survival. *Ann Clin Transl Neurol.* 2018 Nov 20;6(1):46-56.
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68. **Aartsma-Rus A**, Goemans N. A Sequel to the Eteplirsén Saga: Eteplirsén Is Approved in the United States but Was Not Approved in Europe. *Nucleic Acid Ther.* 2018 Dec 11.
69. Kogelman B, Putker K, Hulsker M, Winter CT, van der Weerd L, **Aartsma-Rus A**, van Putten M. Voluntary exercise improves muscle function and does not exacerbate muscle and heart pathology in aged Duchenne muscular dystrophy mice. *J Mol Cell Cardiol.* 2018 Oct 15.
70. Gordish-Dressman H, Willmann R, Dalle Pазze L, Kreibich A, van Putten M, Heydemann A, Bogdanik L, Lutz C, Davies K,

- Demonbruen AR, Duan D, Elsey D, Fukada SI, Girgenrath M, Patrick Gonzalez J, Grounds MD, Nichols A, Partridge T, Passini M, Sanarica F, Schnell FJ, Wells DJ, Yokota T, Young CS, Zhong Z, Spurney C, Spencer M, De Luca A, Nagaraju K, **Aartsma-Rus A**. "Of Mice and Measures": A Project to Improve How We Advance Duchenne Muscular Dystrophy Therapies to the Clinic. *J Neuromuscul Dis*. 2018 Sep 3.
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74. **Aartsma-Rus A**, Mercuri E, Vroom E, Balabanov P. Meeting report of the "Regulatory Exchange Matters" session at the 5th International TREAT-NMD Conference: Lessons in communication: How an early dialogue between patients, regulators and academics can further therapy development for neuromuscular disorders Freiburg, Germany, 27-29 November 2017. *Neuromuscul Disord*. 2018 Apr 20.
75. Pasteuning-Vuhman S, Putker K, Tanganyika-de Winter CL, Boertje-van der Meulen JW, van Vliet L, Overzier M, Plomp JJ, **Aartsma-Rus A**, van Putten M. Natural disease history of the dy2J mouse model of laminin $\alpha 2$ (merosin)-deficient congenital muscular dystrophy. *PLoS One*. 2018 May 15;13(5):e0197388.
76. Spitali P, Hettne K, Tsonaka R, Charrouf M, van den Bergen J, Koeks Z, Kan HE, Hooijmans MT, Roos A, Straub V, Muntoni F, Al-Khalili-Szigyarto C, Koel-Simmelink MJA, Teunissen CE, Lochmüller H, Niks EH, **Aartsma-Rus A**. Tracking disease progression non-invasively in Duchenne and Becker muscular dystrophies. *J Cachexia Sarcopenia Muscle*. 2018 Apr 16.
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ProjectsMembershipsOther Relevant Information