

## Curriculum Vitae

Personal information Elizabeth Vroom

### Work experience

1. Employer: Duchenne Parent Project
  - Start date: 012015
  - End date: 012024
  - Position: CEO
  - Activities: Raising Awareness Fundraising Funding Research Advocacy Improvement of Care Provide Information for families and professionals International collaboration
  - Country: Netherlands
2. Employer: E. Vroom BV
  - Start date: 081983
  - End date: 012015
  - Position: Owner
  - Activities: Dentist/Orthodontist
  - Country: Netherlands

### Education and training

1. Subject:
  - Start date: 091971
  - End date: 051978
  - Qualification: Dentist
  - Organisation: RijksUniversiteit Groningen
  - Country: Netherlands
2. Subject:
  - Start date: 091979
  - End date: 091983
  - Qualification: Specialist Orthodontics and Maxillofacial Orthopedics
  - Organisation: Radboud University, Nijmegen
  - Country: Netherlands

### Additional information

#### Publications

##### *Publications past 7 years*

[Towards harmonization of clinical tools for assessing Brain Involvement in Dystrophinopathies \(BIND\): report of four expert workshops: Newcastle, Leiden, Rome, Paris.](#)

Hendriksen J, Weerkamp P, Miranda R, Kolesnik A, Chieffo D, Skuse D, **Vroom E**, Geagan C, Muntoni F, Mercuri E; BIND WP5 working group. *Neuromuscul Disord.* 2024 Oct 4;44:104452. doi: 10.1016/j.nmd.2024.104452. Online ahead of print. PMID: 39383638

[The Dutch Dystrophinopathy Database: A National Registry with Standardized Patient and Clinician Reported Real-World Data.](#)

van de Velde NM, Krom YD, Bongers J, Hoek RJA, Ikelaar NA, van der Holst M, Naarding KJ, van den Bergen JC, **Vroom E**, Horemans A, Hendriksen JGM, de Groot IJM, Houwen-van Opstal SLS, Verschuuren JJGM, van Duyvenvoorde HA, Snijder RR, Niks EH. *J Neuromuscul Dis.* 2024;11(5):1095-1109. doi: 10.3233/JND-240061. PMID: 39031379

[EURO-NMD registry: federated FAIR infrastructure, innovative technologies and concepts of a patient-centred registry for rare neuromuscular disorders.](#)

Atalaia A, Wandrei D, Lalout N, Thompson R, Tassoni A, 't Hoen PAC, Athanasiou D, Baker SA, Sakellariou P, Paliouras G, D'Angelo C, Horvath R, Mancuso M, van der Beek N, Kornblum C, Kirschner J, Pareyson D, Bassez G, Blacas L, Jacoupy M, Eng C, Lamy F, Plançon JP, Haberlova J, Brusse E, Hoeijmakers JGJ, de Visser M, Claeys KG, Paradas C, Toscano A, Silani V, Gyenge M, Reviers E, Hamroun D, **Vroom E**, Wilkinson MD, Lochmuller H, Evangelista T, Orphanet J. *Rare Dis.* 2024 Feb 14;19(1):66. doi: 10.1186/s13023-024-03059-3. PMID: 38355534

[Moving Beyond the 2018 Minimum International Care Considerations for Osteoporosis Management in Duchenne Muscular Dystrophy \(DMD\): Meeting Report from the 3rd International Muscle-Bone Interactions Meeting 7th and 14th November 2022.](#)

Phung K, Crabtree N, Connolly AM, Furlong P, Hoffman EP, Jackowski SA, Jayash SN, Johnson A, Koujok K, Munns CF, Niks E, Rauch F, Schrader R, Turner C, **Vroom E**, Weber DR, Wong BL, Guglieri M, Ward LM, Wong SC. *J Neuromuscul Dis.* 2024;11(1):233-252. doi: 10.3233/JND-230176. PMID: 37980681

[269th ENMC international workshop: 10 years of clinical trials in Duchennemuscular dystrophy - What have we](#)

[learned? 9-11 December 2022, Hoofddorp, The Netherlands.](#)

Naarding KJ, Stimpson G, Ward SJ, Goemans N, McDonald C, Mercuri E, Muntoni F; 269th ENMC workshop participants. *Neuromuscul Disord.* 2023 Nov;33(11):897-910. doi: 10.1016/j.nmd.2023.10.003. Epub 2023 Oct 11. PMID: 37926638

[Harmonization of outcomes in epidermolysis bullosa: report of the Core Outcome Sets for Epidermolysis Bullosa \(COSEB\) kick-off meeting.](#)

Korte EWH, Spuls PI, van den Akker PC, Kiritsi D, Laimer M, Pasmooij AMG, Riedl R, **Vroom E**, Wally V, Welpone T, Bolling MC. *Br J Dermatol.* 2024 Jan 23;190(2):268-270. doi: 10.1093/bjd/ljad361. PMID: 37792735

[Patient reported outcome measure for upper limb in Duchenne muscular dystrophy: correlation with PUL2.0.](#)

Cicala G, Pane M, Coratti G, Brogna C, Fanelli L, Norcia G, Forcina N, Mazzone E, Stanca G, Ferrante R, Vento A, Ferraroli E, Ricci M, Capasso A, Leone D, Palermo C, Berti B, Cutrona C, Mahyew A, Duong T, Goemans N, **Vroom E**, Mercuri E. *Neuromuscul Disord.* 2023 Sep;33(9):69-73. doi: 10.1016/j.nmd.2023.07.003. Epub 2023 Jul 22. PMID: 37612177

> [The Dilemma of Choice for Duchenne Patients Eligible for Exon 51 Skipping The European Experience.](#)

Aartsma-Rus A, De Waele L, Houwen-Opstal S, Kirschner J, Krom YD, Mercuri E, Niks EH, Straub V, van Duyvenvoorde HA, **Vroom E**. *J Neuromuscul Dis.* 2023;10(3):315-325. doi: 10.3233/JND-221648. PMID: 36911945

> [263rd ENMC International Workshop: Focus on female carriers of dystrophinopathy: refining recommendations for prevention, diagnosis, surveillance, and treatment. Hoofddorp, The Netherlands, 13-15 May 2022.](#)

Sarkozy A, Quinlivan R, Bourke JP, Ferlini A; ENMC 263rd Workshop Study Group. *Neuromuscul Disord.* 2023 Mar;33(3):274-284. doi: 10.1016/j.nmd.2023.01.003. Epub 2023 Jan 12. PMID: 36804616

> [A Comparison of Caregiver and Patient Preferences for Treating Duchenne Muscular Dystrophy.](#)

Crossnohere NL, Fischer R, **Vroom E**, Furlong P, Bridges JFP. *Patient.* 2022 Sep;15(5):577-588. doi: 10.1007/s40271-022-00574-y. Epub 2022 Mar 4. PMID: 35243571

> [The Role of Patient Involvement When Developing Therapies.](#)

Aartsma-Rus A, **Vroom E**, O'Reilly D. *Nucleic Acid Ther.* 2022 Apr;32(2):118-122. doi: 10.1089/nat.2021.0048. Epub 2021 Oct 1. PMID: 34597188 Free PMC article.

> 263rd ENMC International Workshop: Focus on female carriers of dystrophinopathy: refining recommendations for prevention, diagnosis, surveillance, and treatment. Hoofddorp, The Netherlands, 13-15 May 2022. Sarkozy A, Quinlivan R, Bourke JP, Ferlini A; ENMC 263rd Workshop Study Group. *Neuromuscul Disord.* 2023 Jan 12;33(3):274-284. doi: 10.1016/j.nmd.2023.01.003. Online ahead of print. PMID: 36804616

> A Comparison of Caregiver and Patient Preferences for Treating Duchenne Muscular Dystrophy. Crossnohere NL, Fischer R, Vroom E, Furlong P, Bridges JFP. *Patient.* 2022 Sep;15(5):577-588. doi: 10.1007/s40271-022-00574-y. Epub 2022 Mar 4. PMID: 35243571

> The Role of Patient Involvement When Developing Therapies. Aartsma-Rus A, Vroom E, O'Reilly D. *Nucleic Acid Ther.* 2022 Apr;32(2):118-122. doi:10.1089/nat.2021.0048. Epub 2021 Oct 1. PMID: 34597188

> How Patient Organizations Can Drive FAIR Data Efforts to Facilitate Research and Health Care: A Report of the Virtual Second International Meeting on Duchenne Data Sharing, March 3, 2021. van Lin N, Paliouras G, Vroom E, 't Hoen PAC, Roos M. *J Neuromuscul Dis.* 2021;8(6):1097-1108. doi: 10.3233/JND-210721. PMID: 34334415

> 249th ENMC International Workshop: The role of brain dystrophin in muscular dystrophy: Implications for clinical care and translational research, Hoofddorp, The Netherlands, November 29th-December 1st 2019. Hendriksen JGM, Thangarajah M, Kan HE, Muntoni F; ENMC 249th workshop study group. *Neuromuscul Disord.* 2020 Sep;30(9):782-794. doi: 10.1016/j.nmd.2020.08.357. Epub 2020 Aug 15. PMID: 32912717

> Safety issues and harmful pharmacological interactions of nutritional supplements in Duchenne muscular dystrophy: considerations for Standard of Care and emerging virus outbreaks. Boccanegra B, Verhaart IEC, Cappellari O, Vroom E, De Luca A. *Pharmacol Res.* 2020 Aug;158:104917. doi: 10.1016/j.phrs.2020.104917. Epub 2020 May 30. PMID: 32485610 Review.

> The evolution of patient-focused drug development and Duchenne muscular dystrophy. Crossnohere NL, Fischer R, Crossley E, Vroom E, Bridges JF. *Expert Rev Pharmacoecon Outcomes Res.* 2020 Feb;20(1):57-68. doi: 10.1080/14737167.2020.1734454. Epub 2020 Mar 6. PMID: 32098551 Review.

> Meeting on data sharing for Duchenne 21-22 March 2019 Amsterdam, the Netherlands. Verhaart IEC, 't Hoen PAC, Roos M, Vroom E; Workshop Participants. *Neuromuscul Disord.* 2019 Oct;29(10):800-810. doi: 10.1016/j.nmd.2019.08.010. Epub 2019 Aug 28. PMID: 31548100 Review. No abstract available.

> 2nd Workshop on upper-extremity assistive technology for people with Duchenne: Effectiveness and usability of arm supports Irvine, USA, 22nd-23rd January 2018. Janssen MMHP, Lobo-Prat J, Bergsma A, Vroom E; workshop participants. *Neuromuscul Disord.* 2019 Aug;29(8):651-656. doi: 10.1016/j.nmd.2019.07.005. Epub 2019 Jul 25. PMID: 31443952 No abstract available.

> 238th ENMC International Workshop: Updating management recommendations of cardiac dystrophinopathy. Hoofddorp, The Netherlands, 30 November - 2 December 2018. Bourke JP, Guglieri M, Duboc D; ENMC 238th Workshop Study Group. *Neuromuscul Disord.* 2019 Aug;29(8):634-643. doi: 10.1016/j.nmd.2019.06.598. Epub 2019 Jul 1. PMID: 31402197 No abstract available.

> Muscle biopsies in clinical trials for Duchenne muscular dystrophy - Patients' and caregivers' perspective. Verhaart IEC, Johnson A, Thakrar S, Vroom E, De Angelis F, Muntoni F, Aartsma-Rus AM, Niks EH. *Neuromuscul Disord.* 2019 Aug;29(8):576-584. doi: 10.1016/j.nmd.2019.06.004. Epub 2019 Jun 12. PMID: 31378431

> "Be an ambassador for change that you would like to see": a call to action to all stakeholders for co-creation in healthcare and medical research to improve quality of life of people with a neuromuscular disease. Ambrosini A, Quinlivan R, Sansone VA, Meijer I, Schrijvers G, Tibben A, Padberg G, de Wit M, Sterenburg E, Mejat A, Breukel AM, Rataj M, Lochmüller H, Willmann R; 235th ENMC workshop study group. *Orphanet J Rare Dis.* 2019 Jun 7;14(1):126. doi: 10.1186/s13023-019-1103-8. PMID: 31174585

> 236th ENMC International Workshop Bone protective therapy in Duchenne muscular dystrophy: Determining the feasibility and standards of clinical trials Hoofddorp, The Netherlands, 1-3 June 2018. Wong SC, Straub V, Ward LM, Quinlivan R; 236th ENMC workshop participants. *Neuromuscul Disord.* 2019 Mar;29(3):251-259. doi: 10.1016/j.nmd.2019.01.002. Epub 2019 Jan 12. PMID: 30803852 No abstract available.

> *The patient's view on rare disease trial design - a qualitative study.* Gaasterland CMW, van der Weide MCJ, du Prie-Olthof MJ, Donk M, Kaatee MM, Kaczmarek R, Lavery C, Leeson-Beevers K, O'Neill N, Timmis O, van Nederveen V, Vroom E, van der Lee JH. *Orphanet J Rare Dis.* 2019 Feb 7;14(1):31. doi: 10.1186/s13023-019-1002-z.PMID: 30732630

> *A Transition Toolkit for Duchenne Muscular Dystrophy.* Trout CJ, Case LE, Clemens PR, McArthur A, Noritz G, Ritzo M, Wagner KR, Vroom E, Kennedy A. *Pediatrics.* 2018 Oct;142(Suppl 2):S110-S117. doi: 10.1542/peds.2018-0333M.PMID: 30275255

> *Primary Care and Emergency Department Management of the Patient With Duchenne Muscular Dystrophy.* Noritz G, Naprawa J, Apkon SD, Kinnett K, Racca F, Vroom E, Birnkrant DJ. *Pediatrics.* 2018 Oct;142(Suppl 2):S90-S98. doi: 10.1542/peds.2018-0333K.PMID: 30275253

> *The POWER-tool: Recommendations for involving patient representatives in choosing relevant outcome measures during rare disease clinical trial design.* Gaasterland CMW, Jansen-van der Weide MC, Vroom E, Leeson-Beevers K, Kaatee M, Kaczmarek R, Bartels B, van der Pol WL, Roes KCB, van der Lee JH. *Health Policy.* 2018 Dec;122(12):1287-1294. doi: 10.1016/j.healthpol.2018.09.011. Epub 2018 Sep 21.PMID: 30274934

> *Report on the workshop: Meaningful outcome measures for Duchenne muscular dystrophy, London, UK, 30-31 January 2017.* Straub V, Mercuri E; DMD outcome measure study group. *Neuromuscul Disord.* 2018 ug;28(8):690-701. doi: 10.1016/j.nmd.2018.05.013. Epub 2018 Jun 6.PMID: 30033203 No abstract available.

> *Nutrition in Duchenne muscular dystrophy 16-18 March 2018, Zaandam, the Netherlands.* Verhaart IEC, van den Engel-Hoek L, Fiorotto ML, Franken-Verbeek M, Vroom E; workshop participants. *Neuromuscul Disord.* 2018 Aug;28(8):680-689. doi:10.1016/j.nmd.2018.05.004. Epub 2018 May 16.PMID: 29910096 No abstract available.

> *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.

> Aartsma-Rus A, Mercuri E, Vroom E, Balabanov P. *Neuromuscul Disord.* 2018 Jul;28(7):619-623. doi: 10.1016/j.nmd.2018.04.009. Epub 2018 Apr 20.PMID: 29778308 No abstract available.

> *227th ENMC International Workshop:: Finalizing a plan to guarantee quality in translational research for neuromuscular diseases* Heemskerk, Netherlands, 10-11 February 2017. Willmann R, Buccella F, De Luca A, Grounds MD; 227th ENMC workshop study group. *Neuromuscul Disord.* 2018 Feb;28(2):185-192. doi: 10.1016/j.nmd.2017.11.002. Epub 2017 Nov 15.PMID: 29361397 No abstract available.

> *226th ENMC International Workshop:: Towards validated and qualified biomarkers for therapy development for Duchenne muscular dystrophy 20-22 January 2017, Heemskerk, The Netherlands.* Aartsma-Rus A, Ferlini A, McNally EM, Spitali P, Sweeney HL; workshop participants. *Neuromuscul Disord.* 2018 Jan;28(1):77-86. doi:10.1016/j.nmd.2017.10.002. Epub 2017 Oct 26.PMID: 29203356 No abstract available.

> *Development of Exon Skipping Therapies for Duchenne Muscular Dystrophy: A Critical Review and a Perspective on the Outstanding Issues.* Aartsma-Rus A, Straub V, Hemmings R, Haas M, Schlosser-Weber G, Stoyanova-Beninska V, Mercuri E, Muntoni F, Sepodes B, Vroom E, Balabanov P. *Nucleic Acid Ther.* 2017 Oct;27(5):251-259. doi: 10.1089/nat.2017.0682. Epub 2017 Aug 10.PMID: 28796573 Review.

## Projects

WDO/UPPMD was involved in 'Vision\_DMD' a horizon2020 project  
[http://cordis.europa.eu/project/rcn/199721\\_en.html](http://cordis.europa.eu/project/rcn/199721_en.html) Share4Rare This project has received funding from European Union's Horizon 2020 programme under Grant Agreement 780262

Currently WDO/UPPMD is involved in

Trials@home <https://trialsathome.com> IMI funded

BIND <https://bindproject.eu> Brain involvement in Dystrophinopathies H2020

BEAMER <https://beamerproject.eu> Improving Adherence Behavior IMI funded

ERDERA <https://www.ejprarediseases.org/erdera/> Horizon Europe Funding

## Memberships

Chair World Duchenne Organization (former UPPMD) [www.worldduchenne.org](http://www.worldduchenne.org)

## Other Relevant Information

I am a patient representative at EMA

member of PCWP at EMA

member of advisory board DARWIN <https://darwin-eu.org>