



## Curriculum Vitae

Personal information **Christian B. (Kit) Roes**

### Work experience

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1. Employer: Radboud University Medical Centre
  - Start date: 082019
  - End date:
  - Position: Professor of Biostatistics
  - Activities:
  - Country: Netherlands
2. Employer: CBG\_MEB
  - Start date: 032010
  - End date:
  - Position: Senior assessor Methodology & former chair Working Group Methodology
  - Activities:
  - Country: Netherlands
3. Employer: UMC Utrecht
  - Start date: 092016
  - End date: 082019
  - Position: Professor of Clinical Trial Methodology
  - Activities:
  - Country: Netherlands
4. Employer: UMC Utrecht
  - Start date: 042013
  - End date: 092016
  - Position: Director Quality and Patient Safety
  - Activities:
  - Country: Netherlands
5. Employer: UMC Utrecht
  - Start date: 092009
  - End date: 092016
  - Position: Professor of Biostatistics
  - Activities:
  - Country: Netherlands
6. Employer: Schering\_Plough / Formerly Organon
  - Start date: 042002
  - End date: 082009
  - Position: Vice President Global Clinical Information
  - Activities: Global Head of Biostatistics, Data Management, Medical Writing and Contract Management. Includes previous positions as local Head Clinical Information, Director Biometrics.
  - Country: Netherlands
7. Employer: Wageningen University and Research Center
  - Start date: 041997
  - End date: 032002
  - Position: Business Unit Manager Biometry
  - Activities:
  - Country: Netherlands
8. Employer: University of Amsterdam
  - Start date: 021995
  - End date: 031997
  - Position: Senior consultant / Researcher
  - Activities:
  - Country: Netherlands
9. Employer: Organon
  - Start date: 071991
  - End date: 011995
  - Position: Senior statistician
  - Activities:
  - Country: Netherlands
10. Employer: Philips Electronics
  - Start date: 111985
  - End date: 061995
  - Position: Consultant Statistics / Head Industrial Statistics
  - Activities:
  - Country: Netherlands

### Education and training

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1. Subject: Harvard Business School
  - Start date: 122015
  - End date: 122015
  - Qualification: None
  - Organisation: Value Measurement in Healthcare
  - Country: United States
2. Subject: Nyenrode University

- Start date: 012002
  - End date: 122002
  - Qualification: Certificate
  - Organisation: Advanced Management Program
  - Country: Netherlands
3. Subject: University of Amsterdam
- Start date: 091990
  - End date: 111995
  - Qualification: PhD
  - Organisation: Mathematics/Statistics
  - Country: Netherlands
4. Subject: University Nijmegen
- Start date: 081980
  - End date: 111985
  - Qualification: Msc
  - Organisation: Mathematics
  - Country: Netherlands

## Additional information

### Publications

1. van Karnebeek CDM, Müller AR, Benkemoun L, Boussaad I, Cornel MC, IntHout J, de Kort M, de Oliveira Martins S, Prigione A, Rigter T, Roes KCB, Sanchez A, Schipper R, Wilkinson MD, 't Hoen PAC. SIMPATHIC: Accelerating drug repurposing for rare diseases by exploiting SIMilarities in clinical and molecular PATHology. *Mol Genet Metab.* 2025 Apr;144(4):109073. doi: 10.1016/j.ymgme.2025.109073. Epub 2025 Mar 1.
2. Storosum BWC, Cohen SE, Steinz CA, Mattila TK, Welten CC, van den Brink W, Roes K, de Haan L, Denys DAJP, Zantvoord JB. Ethnic differences in efficacy of drug treatment in patients with an acute manic episode: an individual patient data meta-analysis of randomized placebo-controlled trials. *Int J Bipolar Disord.* 2025 Feb 23;13(1):8. doi: 10.1186/s40345-025-00371-0.
3. Jiang C, Beji C, Zebachi S, Hayek GY, Cetinyurek-Yavuz A, Fayyad MBN, Rodwell L, Roes KCB, Amzal B, Gerlinger C, Porcher R, Tanniou J. Decision-Making Criteria and Methods for Initiating Late-Stage Clinical Trials in Drug Development From a Multi-Stakeholder Perspective: A Scoping Review. *Clin Pharmacol Ther.* 2025 Apr;117(4):978-988. doi: 10.1002/cpt.3566.
4. Arlett P, Umuhire D, Verpillat P, Foggi P, Wändel Liminga U, Sepodes B, Lunzer M, Aylward B, Vamvakas S, Roes K, Pétavy F, Thirstrup S, Lamas M, Cooke E, Broich K. Clinical Evidence 2030. *Clin Pharmacol Ther.* 2025 Apr;117(4):884-886. doi: 10.1002/cpt.3596.
5. Daletzakis A, Roes KCB, Jonker MA. Estimation of the Restricted Mean Duration of Response (RMDoR) in Oncology. *Pharm Stat.* 2025 Jan-Feb;24(1):e2468. doi: 10.1002/pst.2468.
6. Cetinyurek Yavuz A, Fayyad MBN, Jiang C, Brion Bouvier F, Beji C, Zebachi S, Hayek GY, Amzal B, Porcher R, Tanniou J, Roes K, Rodwell L. On the Concepts, Methods, and Use of "Probability of Success" for Drug Development Decision-Making: A Scoping Review. *Clin Pharmacol Ther.* 2025 Apr;117(4):967-977. doi: 10.1002/cpt.3571.
7. van Boven JFM, Costello RW, Roes KCB, Brusselle GG, Hansen K, Krishnan JA, Brightling CE, Roche N, Siddiqui S, Kirenga BJ, Pinnock H, Chan AHY. Augmenting clinical trials in asthma through digital technology, decentralised designs, and person-centric endpoints: opportunities and challenges. *Lancet Respir Med.* 2025 Feb;13(2):177-188. doi: 10.1016/S2213-2600(24)00327-8.
8. Storosum BWC, Cohen SE, Mattila TK, Roes KCB, Welten C, van den Brink W, de Haan L, Denys D, Zantvoord JB. Gender differences in the response to antipsychotics or mood stabilizers in patients with acute mania: An individual patient data meta-analysis of placebo-controlled studies. *J Psychiatr Res.* 2025 Jan;181:358-364. doi: 10.1016/j.jpsychires.2024.11.014.
9. Correia Pinheiro L, Arlett P, Roes K, Musuamba Tshinanu F, Westman G, Frias Z, Hamann H, Berenguer Jornet J, Khan I, Larsen J, Broich K, Cooke E. Artificial Intelligence in European Medicines Regulation: From Vision to Action. Harnessing the Capabilities of Artificial Intelligence for the Benefit of Public and Animal Health. *Clin Pharmacol Ther.* 2025 Feb;117(2):335-336. doi: 10.1002/cpt.3494.
10. Chesshyre ELD, Bradbury JD, Cook H, Rocchi F, Roes K, Turner MA, Brüggemann R, Warris A. The impact of the COVID-19 pandemic and the changing landscape of CF on the cASPerCF trial: a real-world experience. *Trials.* 2024 Oct 2;25(1):645. doi: 10.1186/s13063-024-08510-1.
11. Cartwright MJ, Friede T, Lawrence D, May E, Mütze T, Roes K. Stakeholders' Perspectives on Current Issues in Data Monitoring Committees. *Biom J.* 2024 Oct;66(7):e202300384. doi: 10.1002/bimj.202300384.
12. Malizia V, de Vlas SJ, Roes KCB, Giardina F. Revisiting the impact of *Schistosoma mansoni* regulating mechanisms on transmission dynamics using SchiSTOP, a novel modelling framework. *PLoS Negl Trop Dis.* 2024 Sep 20;18(9):e0012464. doi: 10.1371/journal.pntd.0012464.
13. Chu WY, Nijman M, Stegeman R, Breur JMPJ, Jansen NJG, Nijman J, van Loon K, Koomen E, Allegaert K, Benders MJNL, Dorlo TPC, Huitema ADR; CRUCIAL trial consortium. Population Pharmacokinetics and Target Attainment of Allopurinol and Oxypurinol Before, During, and After Cardiac Surgery with Cardiopulmonary Bypass in Neonates with Critical Congenital Heart Disease. *Clin Pharmacokinet.* 2024 Aug;63(8):1205-1220. doi: 10.1007/s40262-024-01401-3.
14. Creemers, Jeroen HA, Ankur Ankan, **Kit CB Roes**, Gijs Schröder, Niven Mehra, Carl G Fidor, I Jolanda M de Vries, and Johannes Textor. 2023. 'In silico cancer immunotherapy trials uncover the consequences of therapy-specific response patterns for clinical trial design and outcome', *Nature Communications*, 14: 2348.
15. Deforth, Manja, Charlotte Micheloud, **Kit C Roes**, and Leonhard Held. 2023. 'Combining evidence from clinical trials in conditional or accelerated approval', *Pharmaceutical Statistics*.
16. Benjamin Hofner, Elina Asikanius, Wolfgang Jacquet, Theodor Framke, Katrien Oude Rengerink, Lukas Aguirre Dávila, Maria Grūnewald, Florian Klinglmüller, Martin Posch, Finbarr P Leacy, Thomas Lang, Armin Koch, Jörg Zinserling, **Kit Roes**. 2023. 'Vaccine development during a pandemic: General lessons for clinical trial design', *Statistics in Biopharmaceutical Research*: 1-13.
17. Janssen Daalen, Jules M, Marjan J Meinders, Federica Giardina, **Kit CB Roes**, Bas C Stunnenberg, Soania Mathur, Philip N Ainslie, Dick HJ Thijssen, and Bastiaan R Bloem. 2022. 'Multiple N-of-1 trials to investigate hypoxia therapy in Parkinson's disease: study rationale and protocol', *BMC neurology*, 22: 1-12.
18. Joosten, Linda PT, Sander van Doorn, Peter M van de Ven, Bart TG Köhlen, Melchior C Nierman, Huiberdina L Koek, Martin EW Hemels, Menno V Huisman, Marieke Kruip, Laura M Faber, Nynke M Wiersma, Wim F Buding, Rob Fijnheer, Henk J Adriaansen, **Kit C Roes**, Arno W Hoes, Frans H Rutten, Geert-Jan Geersing. 2023. 'Safety of switching from a vitamin K antagonist to a non-vitamin K antagonist oral anticoagulant in frail older patients with atrial fibrillation: results of the FRAIL-AF randomized controlled trial', *Circulation*.
19. Kessels, Rob, Anne M May, Miriam Koopman, and **Kit CB Roes**. 2023. 'The Trial within Cohorts (TwICs) study design in oncology: experience and methodological reflections', *BMC Medical Research Methodology*, 23: 1-13.
20. *Pateras, Konstantinos, Kit CB Roes, and Polychronis Kostoulas. 2023. 'Visualizing stability in studies: the moving average meta-analysis (MA2)', arXiv preprint arXiv:2309.13640.*
21. **Roes, Kit**, and Janet Wittes. 2023. '14th Annual University of Pennsylvania Conference on statistical issues in clinical trials/subgroup analysis in clinical trials: Opportunities and challenges (morning panel discussion)', *Clinical Trials*: 17407745231175078.
22. Schootemeijer, Sabine, Nienke M de Vries, Eric A Macklin, **Kit CB Roes**, Hilde Joosten, Larsson Omberg,

- Alberto Ascherio, Michael A Schwarzschild, and Bastiaan R Bloem. 2023. 'The STEPWISE study: study protocol for a smartphone-based exercise solution for people with Parkinson's Disease (randomized controlled trial)', *BMC neurology*, 23: 323.
23. Simons, Michiel, **Kit CB Roes**, Marleen Kets, Barbara M Norquist, and Elizabeth M Swisher. 2023. 'TUBectomy with delayed oophorectomy as an alternative to risk-reducing salpingo-oophorectomy in high-risk women to assess the safety of prevention: the TUBA-WISP II study protocol'.
  24. Rajeshwari Sridhara, Elizabeth Barksdale, Olga Marchenko, Qi Jiang, Yuki Ando, Erick Bloomquist, Michael Coory, Melissa Crouse, Evgeny Degtyarev, Theodor Framke, Boris Freidlin, David E Gerber, Thomas Gwise, Filip Josephson, Lorenzo Hess, Paul Kluetz, Daniel Li, Sumithra Mandrekar, Martin Posch, Khadija Rantell, Bohdana Ratitch, Andrew Raven, **Kit Roes**, Kaspar Rufibach, Sinan B Sarac, Richard Simon, Harpreet Singh, Marc Theoret, Andrew Thomson, Emmanuel Zuber, Yuan Li Shen, Richard Pazdur. 2023. 'Cancer Clinical Trials beyond Pandemic: Report of an American Statistical Association Biopharmaceutical Section Open Forum Discussion', *Statistics in Biopharmaceutical Research*, 15: 444-49.
  25. Marloes Stam, Camiel A Wijngaarde, Bart Bartels, Fay-Lynn Asselman, Louise AM Otto, Laura E Habets, Ruben PA van Eijk, Bas M Middelkoop, H Stephan Goedee, Janke F de Groot, **Kit CB Roes**, Marja AGC Schoenmakers, Edward ES Nieuwenhuis, Inge Cuppen, Leonard H van den Berg, Renske I Wadman, W Ludo van der Pol. 2023. 'Randomized double-blind placebo-controlled crossover trial with pyridostigmine in spinal muscular atrophy types 2-4', *Brain Communications*, 5: fcac324.
  26. Steenbeek, Miranda P, Majke HD van Bommel, Christine B Peterson, Michiel Simons, **Kit CB Roes**, Marleen Kets, Barbara M Norquist, Elizabeth M Swisher, Rosella PMG Hermens, and Karen H Lu. 2023. 'TUBectomy with delayed oophorectomy as an alternative to risk-reducing salpingo-oophorectomy in high-risk women to assess the safety of prevention: the TUBA-WISP II study protocol', *International Journal of Gynecologic Cancer*, 33.
  27. Storoosum, Bram WC, Taina Mattila, Tamar D Wohlfarth, Christine C Gispén-de Wied, **Kit CB Roes**, Wim van den Brink, Lieuwe de Haan, Damiaan AJP Denys, and Jasper B Zantvoord. 2023. 'Gender differences in the response to antipsychotic medication in patients with schizophrenia: An individual patient data meta-analysis of placebo-controlled studies', *Psychiatry research*, 320: 114997.
  28. Storoosum, Bram WC, Cedrine Steinz, Sem E Cohen, Taina Mattila, Wim van den Brink, **Kit Roes**, Lieuwe de Haan, Damiaan AJP Denys, and Jasper B Zantvoord. 2023. 'Ethnic differences in response to atypical antipsychotics in patients with schizophrenia: individual patient data meta-analysis of randomised placebo-controlled registration trials submitted to the Dutch Medicines Evaluation Board', *BJPsych open*, 9: e45.
  29. van Eijk, Ruben PA, Leonard H van den Berg, **Kit CB Roes**, Lu Tian, Tze L Lai, Lorene M Nelson, Chenyu Li, Anna Scowcroft, Jesus Garcia - Segovia, and Ying Lu. 2023. 'Hybrid controlled clinical trials using concurrent registries in Amyotrophic Lateral Sclerosis: A feasibility study', *Clinical Pharmacology & Therapeutics*, 114: 883-92.
  30. van Gelder, Marleen MHJ, Laura Rodwell, and **Kit CB Roes**. 2023. 'Designing observational studies by target trial emulation', *Nederlands Tijdschrift Voor Geneeskunde*, 167: D7702-D02.
  31. Willemsse, Sean W, **Kit CB Roes**, Philip Van Damme, Orla Hardiman, Caroline Ingre, Monica Povedano, Naomi R Wray, Marleen Gijzen, Mirjam S De Pagter, and Koen C Demaeghe. 2022. 'Lithium carbonate in amyotrophic lateral sclerosis patients homozygous for the C-allele at SNP rs12608932 in UNC13A: protocol for a confirmatory, randomized, group-sequential, event-driven, double-blind, placebo-controlled trial', *Trials*, 23: 978
  32. Rajeshwari Sridhara, Olga Marchenko, Qi Jiang, Richard Pazdur, Martin Posch, Mary Redman, Yevgen Tymofeyev, Xiaoyun (Nicole) Li, Marc Theoret, Yuan Li Shen, Thomas Gwise, Lorenzo Hess, Michael Coory, Andrew Raven, Naoto Kotani, Kit Roes, Filip Josephson, Scott Berry, Richard Simon & Bruce Binkowitz (2022). Type I Error Considerations in Master Protocols With Common Control in Oncology Trials: Report of an American Statistical Association Biopharmaceutical Section Open Forum Discussion, *Statistics in Biopharmaceutical Research*, 14:3, 349\_352, DOI: 10.1080/19466315.2021.1906743
  33. Rajeshwari Sridhara, Olga Marchenko, Qi Jiang, Richard Pazdur, Martin Posch, Scott Berry, Marc Theoret, Yuan Li Shen, Thomas Gwise, Lorenzo Hess, Andrew Raven, Khadija Rantell, Kit Roes, Richard Simon, Mary Redman, Yuan Ji & Cindy Lu (2022). Use of Nonconcurrent Common Control in Master Protocols in Oncology Trials: Report of an American Statistical Association Biopharmaceutical Section Open Forum Discussion, *Statistics in Biopharmaceutical Research*, 14:3, 353\_357, DOI: 10.1080/19466315.2021.1938204
  34. Vos JL, Raafs AG, van der Velde N, Germans T, Biesbroek PS, Roes K, Hirsch A, Heymans SRB, Nijveldt R. Comprehensive Cardiovascular Magnetic Resonance-Derived Myocardial Strain Analysis Provides Independent Prognostic Value in Acute Myocarditis. *J Am Heart Assoc*. 2022 Oct 4;11(19):e025106. doi: 10.1161/JAHA.121.025106. Epub 2022 Sep 21. PMID: 36129042. Janssen Daalen JM, Meinders MJ, Giardina F, Roes KCB, Stunnenberg BC, Mathur S, Ainslie PN, Thijssen DHJ, Bloem BR. Multiple N\_of\_1 trials to investigate hypoxia therapy in Parkinson's disease: study rationale and protocol. *BMC Neurol*. 2022 Jul 14;22(1):262. doi: 10.1186/s12883-022-02770-7. PMID: 35836147; PMCID: PMC9281145.
  35. Raafs AG, Vos JL, Henkens MTHM, Slurink BO, Verdonschot JAJ, Bossers D, Roes K, Gerretsen S, Knackstedt C, Hazebroek MR, Nijveldt R, Heymans SRB. Left Atrial Strain Has Superior Prognostic Value to Ventricular Function and Delayed Enhancement in Dilated Cardiomyopathy. *JACC Cardiovasc Imaging*. 2022 Jun;15(6):1015-1026. doi: 10.1016/j.jcmg.2022.01.016. Epub 2022 May 11. PMID: 35680209.
  36. Mitroui M, Teerenstra S, Oude Rengerink K, Pétavy F, Roes KCB. Estimation of treatment effects in short-term depression studies. An evaluation based on the ICH E9(R1) estimands framework. *Pharm Stat*. 2022 Sep;21(5):1037-1057. doi: 10.1002/pst.2214. Epub 2022 Jun 9. PMID: 35678545; PMCID: PMC9543408.
  37. van Eijk RPA, Roes KCB, van den Berg LH, Lu Y. Joint modeling of endpoints can be used to answer various research questions in randomized clinical trials. *J Clin Epidemiol*. 2022 Jul;147:32-39. doi: 10.1016/j.jclinepi. 2022.03.009. Epub 2022 Mar 26. PMID: 35346783.
  38. Mensink FB, Ten Cate TJF, Damen SAJ, Roes K, Di Mario C, Singh V, Ali ZA, Skinner W, Artis A, Torguson R, Zhang C, Doros G, Garcia-Garcia HM, Mintz GS, Geuns RV, Waksman R. Near-infrared spectroscopy predicts events in men and women: Results from the Lipid Rich Plaque study. *Int J Cardiol Heart Vasc*. 2022 Mar 8;39:100985. doi: 10.1016/j.ijcha.2022.100985. PMID: 35281753; PMCID: PMC8914327.
  39. Stegeman R, Nijman M, Breur JMPJ, Groenendaal F, Haas F, Derks JB, Nijman J, van Beynum IM, Taverne YJHJ, Bogers AJJC, Helbing WA, de Boode WP, Bos AF, Berger RMF, Accord RE, Roes KCB, de Wit GA, Jansen NJG, Benders MJNL; CRUCIAL trial consortium. CeRebUm and CardIac Protection with Allopurinol in Neonates with Critical Congenital Heart Disease Requiring Cardiac Surgery with Cardiopulmonary Bypass (CRUCIAL): study protocol of a phase III, randomized, quadruple-blinded, placebo-controlled, Dutch multicenter trial. *Trials*. 2022 Feb 23;23(1):174. doi: 10.1186/s13063-022-06098-y. PMID: 35197082; PMCID: PMC8867620.
  40. Daletzakis A, van den Bor R, Jonker MA, Roes KCB, van Tinteren H. Estimation and expected sample size in Simon's two stage designs that stop as early as possible. *Pharm Stat*. 2022 Feb 22. doi: 10.1002/pst.2200. Epub ahead of print. PMID: 35191174.
  41. Kliet T, Van Eijk RPA, Al-Chalabi A, Albanese A, Andersen PM, Amador MDM, Bråthen G, Brunaud Danel V, Brylev L, Camu W, De Carvalho M, Cereda C, Cetin H, Chaverri D, Chiò A, Corcia P, Couratier P, De Marchi F, Desnuelle C, Van Es MA, Esteban J, Filosto M, García Redondo A, Grosskreutz J, Hanemann CO, Holmøy T, Høyer H, Ingre C, Koritnik B, Kuzma-Kozakiewicz M, Lambert T, Leigh PN, Lunetta C, Mandrioli J, Mcdermott CJ, Meyer T, Mora JS, Petri S, Povedano M, Reviers E, Riva N, Roes KCB, Rubio MÁ, Salachas F, Sarafov S, Sorarú G, Stevic Z, Svenstrup K, Møller AT, Turner MR, Van Damme P, Van Leeuwen LAG, Varona L, Vázquez Costa JF, Weber M, Hardiman O, Van Den Berg LH. Clinical trials in pediatric ALS: a TRICALS feasibility study. *Amyotroph Lateral Scler Frontotemporal Degener*. 2022 Feb 16:1-8. doi: 10.1080/21678421.2021.2024856. Epub ahead of print. PMID: 35172656.
  42. van Eijk RPA, Roes KCB, de Greef van der Sandt I, van den Berg LH, Lu Y. Functional Loss and Mortality in Randomized Clinical Trials for Amyotrophic Lateral Sclerosis: To Combine, or ot to Combine\_That is the Estimand. *Clin Pharmacol Ther*. 2022 Jan 25. doi: 10.1002/cpt.2533. Epub ahead of print. PMID:

35076930.

43. van Poelgeest R, Schrijvers A, Boonstra A, Roes K. Medical Specialists' Perspectives on the Influence of Electronic Medical Record Use on the Quality of Hospital Care: Semistructured Interview Study. *JMIR Hum Factors*. 2021 Oct 27;8(4):e27671. doi: 10.2196/27671. PMID: 34704955.
44. van Eijk RPA, Beelen A, Kruitwagen ET, Murray D, Radakovic R, Hobson E, Knox L, Helleman J, Burke T, Rubio Pérez MA, Reviers E, Genge A, Steyn FJ, Ngo S, Eaglesham J, Roes KCB, van den Berg LH, Hardiman O, McDermott CJ. A Road Map for Remote Digital Health Technology for Motor Neuron Disease. *J Med Internet Res*. 2021 Sep 22;23(9):e28766. doi: 10.2196/28766. PMID: 34550089.
45. Müller AR, Zinkstok JR, Rommelse NNJ, van de Ven PM, Roes KCB, Wijburg FA, de Rooij\_Askes E, Linders C, Boot E, van Eeghen AM. Methylphenidate for attention\_deficit/hyperactivity disorder in patients with Smith\_Magenis syndrome: protocol for a series of N\_of\_1 trials. *Orphanet J Rare Dis*. 2021 Sep 8;16(1):380. doi:10.1186/s13023\_021\_02003\_z. PMID: 34496899; PMCID: PMC8424817.
46. van Eijk RPA, Nikolakopoulos S, Roes KCB, Kendall L, Han SS, Lavrov A, Epstein N, Kliet T, de Jongh AD, Westeneng HJ, Al\_Chalabi A, Van Damme P, Hardiman O, Shaw PJ, McDermott CJ, Eijkemans MJC, van den Berg LH. Innovating Clinical Trials for Amyotrophic Lateral Sclerosis: Challenging the Established Order. *Neurology*. 2021 Sep 14;97(11):528\_536. doi: 10.1212/WNL.0000000000012545. Epub 2021 Jul 27. PMID: 34315786; PMCID: PMC8456357.
47. Kersten RFMR, Öner FC, Arts MP, Mitroiu M, Roes KCB, de Gast A, van Gaalen SM. Letter to the editor regarding "Two\_year results of a double\_blind multicenter randomized controlled non\_inferiority trial of polyetheretherketone (PEEK) versus silicon nitride spinal fusion cages in patients with symptomatic degenerative lumbar disc disorders". *J Spine Surg*. 2021 Jun;7(2):249\_251. doi:10.21037/jss\_21\_6. PMID: 34296041; PMCID: PMC8261572.
48. Sarrami\_Foroushani A, Lassila T, MacRaild M, Asquith J, Roes KCB, Byrne JV, Frangi AF. In\_silico trial of intracranial flow diverters replicates and expands insights from conventional clinical trials. *Nat Commun*. 2021 Jun 23;12(1):3861. doi: 10.1038/s41467\_021\_23998\_w. PMID: 34162852; PMCID: PMC8222326.
49. Pateras K, Nikolakopoulos S, Roes KCB. Combined assessment of early and late\_phase outcomes in orphan drug development. *Stat Med*. 2021 May 30;40(12):2957\_2974. doi: 10.1002/sim.8952. Epub 2021 Apr 4. PMID: 33813759; PMCID: PMC8252448.
50. van Eijk RPA, de Jongh AD, Nikolakopoulos S, McDermott CJ, Eijkemans MJC, Roes KCB, van den Berg LH. An old friend who has overstayed their welcome: the ALSFRS\_R total score as primary endpoint for ALS clinical trials. *Amyotroph Lateral Scler Frontotemporal Degener*. 2021 May;22(3\_4):300\_307. doi: 10.1080/21678421.2021.1879865. Epub 2021 Feb 2. PMID: 33527843.
51. Müller AR, Brands MMMG, van de Ven PM, Roes KCB, Cornel MC, van Karnebeek CDM, Wijburg FA, Daams JG, Boot E, van Eeghen AM. Systematic Review of N\_of\_1 Studies in Rare Genetic Neurodevelopmental Disorders: The Power of 1. *Neurology*. 2021 Mar 16;96(11):529\_540. doi: 10.1212/WNL.0000000000011597. Epub 2021 Jan 27. PMID: 33504638; PMCID: PMC8032375.
52. Ramjith J, Roes KCB, Zar HJ, Jonker MA. Flexible modelling of risk factors on the incidence of pneumonia in young children in South Africa using piece\_wise exponential additive mixed modelling. *BMC Med Res Methodol*. 2021 Jan 11;21(1):17. doi: 10.1186/s12874\_020\_01194\_6. PMID: 33430789; PMCID: PMC7802241.
53. Kersten RFMR, Öner FC, Arts MP, Mitroiu M, Roes KCB, de Gast A, van Gaalen SM. The SNAP Trial: 2\_Year Results of a Double\_Blind Multicenter Randomized Controlled Trial of a Silicon Nitride Versus a PEEK Cage in Patients After Lumbar Fusion Surgery. *Global Spine J*. 2021 Jan 7:2192568220985472. doi: 10.1177/2192568220985472. Epub ahead of print. PMID: 33406905.
54. Maas RPPWM, Ramjith J, Klockgether T, Roes KCB, van de Warrenburg BPC. A word of hope for ataxia trials in COVID\_19 time and beyond. *J Neurol*. 2021 Jul;268(7):2343\_2345. doi: 10.1007/s00415\_020\_10231\_9. Epub 2020 Sep 22. PMID: 32964331; PMCID: PMC7508233.
55. Pateras K, Nikolakopoulos S, Roes KCB. Prior distributions for variance parameters in a sparse\_event meta\_analysis of a few small trials. *Pharm Stat*. 2021 Jan;20(1):39\_54. doi: 10.1002/pst.2053. Epub 2020 Aug 6. PMID: 32767452; PMCID: PMC7818503.
56. Mitroiu M, Oude Rengerink K, Teerenstra S, Pétavy F, Roes KCB. A narrative review of estimands in drug development and regulatory evaluation: old wine in new barrels? *Trials*. 2020 Jul 23;21(1):671. doi: 10.1186/s13063\_020\_04546\_1. PMID: 32703247; PMCID: PMC7376663.
57. van Eijk RPA, Kliet T, McDermott CJ, Roes KCB, Van Damme P, Chio A, Weber M, Ingre C, Corcia P, Povedano M, Reviers E, van Es MA, Al\_Chalabi A, Hardiman O, van den Berg LH. TRICALS: creating a highway toward a cure. *Amyotroph Lateral Scler Frontotemporal Degener*. 2020 Nov;21(7\_8):496\_501. doi: 10.1080/21678421.2020.1788092. Epub 2020 Jul 9. PMID: 32643415.
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## Projects

### Current involvement as Work Package and/or Tasks lead:

Horizon Europe: (1) MORE-Europa: More Effectively Using Registries to Support Patient Centered Regulatory and Health Technology Assessment decision making. (2) SIMPATHIC: Innovative approach to identify and develop targeted (re-purposed) drugs for rare diseases with shared pathophysiological mechanisms. (3) INVENTS: Innovative designs, extrapolation, simulation methods and evidence tools for rare diseases addressing regulatory needs (4) REGolution: Marie Skłodowska-Curie Doctoral Network to advance quantitative methodology for regulatory decision making and strengthen the competencies in this field in European Medicines Regulatory Network.

IHI funded: Realised: Developing operational and methodological tools and resources that can dramatically advance the clinical evaluation of new treatments for rare and ultra rare diseases.

**Current involvement in advisory role:** (1) ERDERA. (2) EU TRICALS Consortium for clinical trials in Amyotrophic Lateral Sclerosis.

**Past project:** Principal Investigator of EU FP 7 project on Methodology for clinical trials in small populations (Asterix project; completed 2017).

## Memberships

Chair EMA Methodology Working Party.

Member Steering Committee Platform for European Regulatory Science Research.

Member Regulatory Science Network Netherlands.

Member IRDiRC Taskforce on Methodology for Clinical Trials in Rare diseases.

Member/chair of multiple independent Data Monitoring Committees, primarily for academically initiated trials. It included iDMC Member for Corcept Therapeutics (ALS, until March 2025) and currently includes iDMC Chair for one trial of F. Hoffman-La Roche (Huntington's disease, current).

## Other Relevant Information