Universiteit Leiden
The Netherlands

Statistical modelling of competing risks with incomplete data: with applications to allogeneic stem cell transplantation

Bonneville, E.F.

Citation
Bonneville, E. F. (2025, July 2). *Statistical modelling of competing risks with incomplete data: with applications to allogeneic stem cell transplantation*. Retrieved from https://hdl.handle.net/1887/4252266

Version:	Publisher's Version
License:	Licence agreement concerning inclusion of doctoral thesis in the Institutional Repository of the University of Leiden
Downloaded from:	https://hdl.handle.net/1887/4252266

Note: To cite this publication please use the final published version (if applicable).

Bibliography

Aalen, O., Borgan, O. and Gjessing, H. (2008) *Survival and Event History Analysis: A Process Point of View.* DOI: 10.1007/978-0-387-68560-1.

Aalen, O. O. and Johansen, S. (1978) An Empirical Transition Matrix for Non-Homogeneous Markov Chains Based on Censored Observations. *Scandinavian Journal of Statistics*, **5**, 141–150. Available at: https://www.jstor.org/stable/4615704.

Acosta-Medina, A. A., Baranwal, A., Johnson, I. M., et al. (2023) Comparison of Pretransplantation Prediction Models for Nonrelapse Mortality in Patients with Myelofibrosis Undergoing Allogeneic Stem Cell Transplantation. *Transplantation and Cellular Therapy*, **29**, 360.e1–360.e8. DOI: 10.1016/j.jtct.2023.02.002.

Adès, L., Itzykson, R. and Fenaux, P. (2014) Myelodysplastic syndromes. *The Lancet*, **383**, 2239–2252. DOI: 10.1016/S0140-6736(13)61901-7.

Allignol, A. and Beyersmann, J. (2010) Software for fitting nonstandard proportional subdistribution hazards models. *Biostatistics*, **11**, 674–675. DOI: 10.1093/biostatistics/kxq018.

Andersen, P. K. and Keiding, N. (2012) Interpretability and importance of functionals in competing risks and multistate models. *Statistics in Medicine*, **31**, 1074–1088. DOI: 10.1002/sim.4385.

Andersen, P. K. and Ravn, H. (2023) *Models for Multi-State Survival Data: Rates, Risks, and Pseudo-Values.*

Andersen, P. K., Abildstrom, S. Z. and Rosthøj, S. (2002) Competing risks as a multi-state model. *Statistical Methods in Medical Research*, **11**, 203–215. DOI: 10.1191/0962280202sm281ra.

Andersen, P. K., Geskus, R. B., de Witte, T., et al. (2012) Competing risks in epidemiology: Possibilities and pitfalls. *International Journal of Epidemiology*, **41**, 861–870.

DOI: 10.1093/ije/dyr213.

Andrinopoulou, E.-R., Rizopoulos, D., Geleijnse, M. L., et al. (2015) Dynamic prediction of outcome for patients with severe aortic stenosis: Application of joint models for longitudinal and time-to-event data. *BMC Cardiovascular Disorders*, **15**, 28. DOI: 10.1186/s12872-015-0035-z.

Antunes, L., Mendonça, D., Bento, M. J., et al. (2021) Dealing with missing information on covariates for excess mortality hazard regression models – Making the imputation model compatible with the substantive model. *Statistical Methods in Medical Research*, **30**, 2256–2268. DOI: 10.1177/09622802211031615.

Archer, L., Koshiaris, C., Lay-Flurrie, S., et al. (2022) Development and external validation of a risk prediction model for falls in patients with an indication for antihypertensive treatment: Retrospective cohort study. *BMJ*, **379**, e070918. DOI: 10.1136/bmj-2022-070918.

Armand, P., Gibson, C. J., Cutler, C., et al. (2012) A disease risk index for patients undergoing allogeneic stem cell transplantation. *Blood*, **120**, 905–913. DOI: 10.1182/blood-2012-03-418202.

Armand, P., Kim, H. T., Logan, B. R., et al. (2014) Validation and refinement of the Disease Risk Index for allogeneic stem cell transplantation. *Blood*, **123**, 3664–3671. DOI: 10.1182/blood-2014-01-552984.

Austin, P. C. (2024) Multiple imputation with competing risk outcomes. *Computational Statistics*. DOI: 10.1007/s00180-024-01518-w.

Austin, P. C., Steyerberg, E. W. and Putter, H. (2021) Fine-Gray subdistribution hazard models to simultaneously estimate the absolute risk of different event types: Cumulative total failure probability may exceed 1. *Statistics in Medicine*, **40**, 4200–4212. DOI: 10.1002/sim.9023.

Austin, P. C., Giardiello, D. and van Buuren, S. (2023) Impute-then-exclude versus exclude-then-impute: Lessons when imputing a variable used both in cohort creation and as an independent variable in the analysis model. *Statistics in Medicine*, **42**, 1525–1541. DOI: 10.1002/sim.9685.

Baker, W. L., Moore, T., Baron, E., et al. (2024) A Systematic Review of Reporting and Handling of Missing Data in Observational Studies Using the UNOS Database. *The Journal of Heart and Lung Transplantation*. DOI: 10.1016/j.healun.2024.10.023.

Bakoyannis, G., Siannis, F. and Touloumi, G. (2010) Modelling competing risks

data with missing cause of failure. *Statistics in Medicine*, **29**, 3172–3185. DOI: 10.1002/sim.4133.

Bartlett, J. W. and Taylor, J. M. G. (2016) Missing covariates in competing risks analysis. *Biostatistics*, **17**, 751–763. DOI: 10.1093/biostatistics/kxw019.

Bartlett, J. W., Seaman, S. R., White, I. R., et al. (2015) Multiple imputation of covariates by fully conditional specification: Accommodating the substantive model. *Statistical Methods in Medical Research*, **24**, 462–487. DOI: 10.1177/0962280214521348.

Bartlett, J. W., Keogh, R. H. and Bonneville, E. F. (2022) *Smcfcs: Multiple Imputation of Covariates by Substantive Model Compatible Fully Conditional Specification*. Manual.

Beesley, L. J., Bartlett, J. W., Wolf, G. T., et al. (2016) Multiple imputation of missing covariates for the Cox proportional hazards cure model. *Statistics in Medicine*, **35**, 4701–4717. DOI: 10.1002/sim.7048.

Bell, M. L., Fiero, M., Horton, N. J., et al. (2014) Handling missing data in RCTs; a review of the top medical journals. *BMC Medical Research Methodology*, **14**, 118. DOI: 10.1186/1471-2288-14-118.

Bellach, A., Kosorok, M. R., Rüschendorf, L., et al. (2019) Weighted NPMLE for the Subdistribution of a Competing Risk. *Journal of the American Statistical Association*, **114**, 259–270. DOI: 10.1080/01621459.2017.1401540.

Bellucci, R., Alyea, E. P., Weller, E., et al. (2002) Immunologic effects of prophylactic donor lymphocyte infusion after allogeneic marrow transplantation for multiple myeloma. *Blood*, **99**, 4610–4617. DOI: 10.1182/blood.V99.12.4610.

Beyersmann, J., Latouche, A., Buchholz, A., et al. (2009) Simulating competing risks data in survival analysis. *Statistics in Medicine*, **28**, 956–971. DOI: 10.1002/sim.3516.

Beyersmann, J., Allignol, A. and Schumacher, M. (2012) *Competing Risks and Multistate Models with R*. Use R! DOI: 10.1007/978-1-4614-2035-4.

Blake, H. A., Leyrat, C., Mansfield, K. E., et al. (2020) Estimating treatment effects with partially observed covariates using outcome regression with missing indicators. *Biometrical Journal*, **62**, 428–443. DOI: 10.1002/bimj.201900041.

Bonneville, E. F., Resche-Rigon, M., Schetelig, J., et al. (2022) Multiple imputation for cause-specific Cox models: Assessing methods for estimation and prediction. *Statistical Methods in Medical Research*, **31**, 1860–1880. DOI: 10.1177/09622802221102623.

Bonneville, E. F., Schetelig, J., Putter, H., et al. (2023) Handling missing covariate data in clinical studies in haematology. *Best Practice & Research Clinical Haematology*, **36**, 101477. DOI: 10.1016/j.beha.2023.101477.

Bonneville, E. F., de Wreede, L. C. and Putter, H. (2024) Why you should avoid using multiple Fine–Gray models: Insights from (attempts at) simulating proportional subdistribution hazards data. *Journal of the Royal Statistical Society Series A: Statistics in Society*, qnae056. DOI: 10.1093/jrsssa/qnae056.

Borgan, Ø. and Keogh, R. (2015) Nested case–control studies: Should one break the matching? *Lifetime Data Analysis*, **21**, 517–541. DOI: 10.1007/s10985-015-9319-y.

Bosch, M., Dhadda, M., Hoegh-Petersen, M., et al. (2012) Immune reconstitution after anti-thymocyte globulin-conditioned hematopoietic cell transplantation. *Cytotherapy*, **14**, 1258–1275. DOI: 10.3109/14653249.2012.715243.

Boulesteix, A.-L., Lauer, S. and Eugster, M. J. A. (2013) A Plea for Neutral Comparison Studies in Computational Sciences. *PLOS ONE*, **8**, e61562. DOI: 10.1371/journal.pone.0061562.

Boulesteix, A.-L., Hoffmann, S., Charlton, A., et al. (2020) A Replication Crisis in Methodological Research? *Significance*, **17**, 18–21. DOI: 10.1111/1740-9713.01444.

Breslow, N. E. (1972) Contribution to discussion of paper by D. R. Cox. *Journal of the Royal Statistical Society, Series B*, **34**, 216–217.

Burton, A. and Altman, D. G. (2004) Missing covariate data within cancer prognostic studies: A review of current reporting and proposed guidelines. *British Journal of Cancer*, **91**, 4–8. DOI: 10.1038/sj.bjc.6601907.

Busca, A. and Aversa, F. (2017) In-vivo or ex-vivo T cell depletion or both to prevent graft-versus-host disease after hematopoietic stem cell transplantation. *Expert Opinion on Biological Therapy*, **17**, 1401–1415. DOI: 10.1080/14712598.2017.1369949.

Carpenter, J. R. and Smuk, M. (2021) Missing data: A statistical framework for practice. *Biometrical Journal*, **63**, 915–947. DOI: 10.1002/bimj.202000196.

Carpenter, J. R., Bartlett, J. W., Morris, T. P., et al. (2023) *Multiple Imputation and Its Application.* 2nd ed. Statistics in practice series.

Carroll, O. U., Morris, T. P. and Keogh, R. H. (2020) How are missing data in covariates handled in observational time-to-event studies in oncology? A systematic review. *BMC Medical Research Methodology*, **20**, 134. DOI: 10.1186/s12874-020-01018-7.

Chiang, C. L. (1961) A Stochastic Study of the Life Table and Its Applications. III. The Follow-Up Study with the Consideration of Competing Risks. *Biometrics*, **17**, 57–78. DOI: 10.2307/2527496.

Clements, L. Z. (2022) *Multiple imputation of a derived variable in a survival analysis context*. PhD thesis.

Clift, A. K., Coupland, C. A. C., Keogh, R. H., et al. (2020) Living risk prediction algorithm (QCOVID) for risk of hospital admission and mortality from coronavirus 19 in adults: National derivation and validation cohort study. *BMJ*, **371**, m3731. DOI: 10.1136/bmj.m3731.

Cornfield, J. (1957) Estimation of the Probability of Developing a Disease in the Presence of Competing Risks. *American Journal of Public Health and the Nations Health*, **47**, 601–607. DOI: 10.2105/ajph.47.5.601.

Cox, D. R. (1972) Regression Models and Life-Tables. *Journal of the Royal Statistical Society: Series B (Methodological)*, **34**, 187–202. DOI: 10.1111/j.2517-6161.1972.tb00899.x.

Cox, D. R. (1975) Partial likelihood. *Biometrika*, **62**, 269–276. DOI: 10.1093/biomet/62.2.269.

D'Amico, G., Morabito, A., D'Amico, M., et al. (2018) Clinical states of cirrhosis and competing risks. *Journal of Hepatology*, **68**, 563–576. DOI: 10.1016/j.jhep.2017.10.020.

de Wreede, L. C., Fiocco, M. and Putter, H. (2010) The mstate package for estimation and prediction in non- and semi-parametric multi-state and competing risks models. *Computer Methods and Programs in Biomedicine*, **99**, 261–274. DOI: 10.1016/j.cmpb.2010.01.001.

de Wreede, L. C., Fiocco, M. and Putter, H. (2011) Mstate: An R Package for the Analysis of Competing Risks and Multi-State Models. *Journal of Statistical Software*, **38**, 1–30. DOI: 10.18637/jss.v038.i07.

Dekker, L., de Koning, C., Lindemans, C., et al. (2020) Reconstitution of T Cell Subsets Following Allogeneic Hematopoietic Cell Transplantation. *Cancers*, **12**, 1974. DOI: 10.3390/cancers12071974.

Delgado, J., Pereira, A., Villamor, N., et al. (2014) Survival analysis in hematologic malignancies: Recommendations for clinicians. *Haematologica*, **99**, 1410–1420. DOI: 10.3324/haematol.2013.100784.

Delord, M. and Génin, E. (2016) Multiple imputation for competing risks regression

with interval-censored data. *Journal of Statistical Computation and Simulation*, **86**, 2217–2228. DOI: 10.1080/00949655.2015.1106543.

Demirtas, H. and Hedeker, D. (2016) Computing the Point-biserial Correlation under Any Underlying Continuous Distribution. *Communications in Statistics - Simulation and Computation*, **45**, 2744–2751. DOI: 10.1080/03610918.2014.920883.

Donders, A. R. T., van der Heijden, G. J. M. G., Stijnen, T., et al. (2006) Review: A gentle introduction to imputation of missing values. *Journal of Clinical Epidemiology*, **59**, 1087–1091. DOI: 10.1016/j.jclinepi.2006.01.014.

Donoghoe, M. W. and Gebski, V. (2017) The importance of censoring in competing risks analysis of the subdistribution hazard. *BMC Medical Research Methodology*, **17**, 52. DOI: 10.1186/s12874-017-0327-3.

Du, H., Alacam, E., Mena, S., et al. (2022) Compatibility in imputation specification. *Behavior Research Methods*. DOI: 10.3758/s13428-021-01749-5.

Dunbar, E. M., Buzzeo, M. P., Levine, J. B., et al. (2008) The relationship between circulating natural killer cells after reduced intensity conditioning hematopoietic stem cell transplantation and relapse-free survival and graft-versus-host disease. *Haematologica*, **93**, 1852–1858. DOI: 10.3324/haematol.13033.

Eefting, M., Halkes, C. J. M., de Wreede, L. C., et al. (2014) Myeloablative T cell-depleted alloSCT with early sequential prophylactic donor lymphocyte infusion is an efficient and safe post-remission treatment for adult ALL. *Bone Marrow Transplantation*, **49**, 287–291. DOI: 10.1038/bmt.2013.111.

Eefting, M., de Wreede, L. C., Halkes, C. J. M., et al. (2016) Multi-state analysis illustrates treatment success after stem cell transplantation for acute myeloid leukemia followed by donor lymphocyte infusion. *Haematologica*, **101**, 506–514. DOI: 10.3324/haematol.2015.136846.

Elfeky, R., Lazareva, A., Qasim, W., et al. (2019) Immune reconstitution following hematopoietic stem cell transplantation using different stem cell sources. *Expert Review of Clinical Immunology*, **15**, 735–751. DOI: 10.1080/1744666X.2019.1612746.

Enders, C. K. (2022) *Applied Missing Data Analysis, 2nd Ed.* Applied missing data analysis, 2nd ed.

Erler, N. S., Rizopoulos, D., Rosmalen, J. van, et al. (2016) Dealing with missing covariates in epidemiologic studies: A comparison between multiple imputation and a full Bayesian approach. *Statistics in Medicine*, **35**, 2955–2974. DOI: 10.1002/sim.6944.

Erler, N. S., Rizopoulos, D. and Lesaffre, E. M. E. H. (2021) JointAI: Joint Analysis and Imputation of Incomplete Data in R. *Journal of Statistical Software*, **100**, 1–56. DOI: 10.18637/jss.v100.i20.

Falcaro, M., Nur, U., Rachet, B., et al. (2015) Estimating Excess Hazard Ratios and Net Survival When Covariate Data Are Missing: Strategies for Multiple Imputation. *Epidemiology*, **26**, 421–428. DOI: 10.1097/EDE.0000000000000283.

Falkenburg, J. H. F. and Jedema, I. (2017) Graft versus tumor effects and why people relapse. *Hematology*, **2017**, 693–698. DOI: 10.1182/asheducation-2017.1.693.

Falkenburg, J. H. F., Schmid, C., Kolb, H. J., et al. (2019) Delayed transfer of immune cells or the art of donor lymphocyte infusion. *The EBMT Handbook: Hematopoietic Stem Cell Transplantation and Cellular Therapies*, 443–448. DOI: 10.1007/978-3-030-02278-5_59.

Fine, J. P. and Gray, R. J. (1999) A Proportional Hazards Model for the Subdistribution of a Competing Risk. *Journal of the American Statistical Association*, **94**, 496–509. DOI: 10.1080/01621459.1999.10474144.

Fleischhauer, K., Shaw, B. E., Gooley, T., et al. (2012) Effect of T-cell-epitope matching at HLA-DPB1 in recipients of unrelated-donor haemopoietic-cell transplantation: A retrospective study. *The Lancet Oncology*, **13**, 366–374. DOI: 10.1016/S1470-2045(12)70004-9.

Gasparini, A. (2018) Rsimsum: Summarise results from Monte Carlo simulation studies. *Journal of Open Source Software*, **3**, 739. DOI: 10.21105/joss.00739.

Gerds, T. A., Scheike, T. H. and Andersen, P. K. (2012) Absolute risk regression for competing risks: Interpretation, link functions, and prediction. *Statistics in Medicine*, **31**, 3921–3930. DOI: 10.1002/sim.5459.

Geskus, R. B. (2011) Cause-Specific Cumulative Incidence Estimation and the Fine and Gray Model Under Both Left Truncation and Right Censoring. *Biometrics*, **67**, 39–49. DOI: 10.1111/j.1541-0420.2010.01420.x.

Geskus, R. B. (2024) Competing Risks: Concepts, Methods, and Software. *Annual Review of Statistics and Its Application*, **11**, 227–254. DOI: 10.1146/annurev-statistics-040522-094556.

Gooptu, M., Kim, H. T., Howard, A., et al. (2019) Effect of Sirolimus on Immune Reconstitution Following Myeloablative Allogeneic Stem Cell Transplantation: An Ancillary Analysis of a Randomized Controlled Trial Comparing Tacrolimus/Sirolimus

and Tacrolimus/Methotrexate (Blood and Marrow Transplant Clinical Trials Network/BMT CTN 0402). *Biology of Blood and Marrow Transplantation*, **25**, 2143–2151. DOI: 10.1016/j.bbmt.2019.06.029.

Gorgi Zadeh, S., Behning, C. and Schmid, M. (2022) An imputation approach using subdistribution weights for deep survival analysis with competing events. *Scientific Reports*, **12**, 3815. DOI: 10.1038/s41598-022-07828-7.

Grambauer, N., Schumacher, M. and Beyersmann, J. (2010) Proportional subdistribution hazards modeling offers a summary analysis, even if misspecified. *Statistics in Medicine*, **29**, 875–884. DOI: 10.1002/sim.3786.

Gray, R. J. (1988) A Class of K-Sample Tests for Comparing the Cumulative Incidence of a Competing Risk. *The Annals of Statistics*, **16**, 1141–1154. DOI: 10.1214/aos/1176350951.

Groenwold, R. H. H., White, I. R., Donders, A. R. T., et al. (2012) Missing covariate data in clinical research: When and when not to use the missing-indicator method for analysis. *CMAJ*, **184**, 1265–1269. DOI: 10.1503/cmaj.110977.

Guillaume, T., Gaugler, B., Chevallier, P., et al. (2012) Escalated lymphodepletion followed by donor lymphocyte infusion can induce a graft-versus-host response without overwhelming toxicity. *Bone Marrow Transplantation*, **47**, 1112–1117. DOI: 10.1038/bmt.2011.231.

Haensch, A.-C., Bartlett, J. and Weiß, B. (2022) Multiple imputation of partially observed covariates in discrete-time survival analysis. *Sociological Methods & Research*, 00491241221140147. DOI: 10.1177/00491241221140147.

Haller, B. and Ulm, K. (2014) Flexible simulation of competing risks data following prespecified subdistribution hazards. *Journal of Statistical Computation and Simulation*, **84**, 2557–2576. DOI: 10.1080/00949655.2013.793345.

Han, S. Y., Mrozek K., Voutsinas J., et al. (2021) Secondary cytogenetic abnormalities in core-binding factor AML harboring inv(16) vs t(8;21). *Blood Advances*, **5**, 2481–2489. DOI: 10.1182/BLOODADVANCES.2020003605.

Hansen, D. K., Kim J., Thompson Z., et al. (2021) ELN 2017 Genetic Risk Stratification Predicts Survival of Acute Myeloid Leukemia Patients Receiving Allogeneic Hematopoietic Stem Cell Transplantation. *Transplantation and Cellular Therapy*, **27**, 256.e1–256.e7. DOI: 10.1016/j.jtct.2020.12.021.

Hassan, N., Eldershaw, S., Stephens, C., et al. (2022) CMV reactivation initiates

long-term expansion and differentiation of the NK cell repertoire. *Frontiers in Immunology*, **13**. DOI: 10.3389/fimmu.2022.935949.

Hayati Rezvan, P., Lee, K. J. and Simpson, J. A. (2015) The rise of multiple imputation: A review of the reporting and implementation of the method in medical research. *BMC Medical Research Methodology*, **15**, 30. DOI: 10.1186/s12874-015-0022-1.

Heitjan, D. F. and Rubin, D. B. (1991) Ignorability and Coarse Data. *The Annals of Statistics*, **19**, 2244–2253. DOI: 10.1214/aos/1176348396.

Hickey, G. L., Philipson, P., Jorgensen, A., et al. (2016) Joint modelling of time-to-event and multivariate longitudinal outcomes: Recent developments and issues. *BMC Medical Research Methodology*, **16**, 117. DOI: 10.1186/s12874-016-0212-5.

Hickey, G. L., Philipson, P., Jorgensen, A., et al. (2018) A Comparison of Joint Models for Longitudinal and Competing Risks Data, with Application to an Epilepsy Drug Randomized Controlled Trial. *Journal of the Royal Statistical Society Series A: Statistics in Society*, **181**, 1105–1123. DOI: 10.1111/rssa.12348.

Hinchliffe, S. R. and Lambert, P. C. (2013) Flexible parametric modelling of cause-specific hazards to estimate cumulative incidence functions. *BMC Medical Research Methodology*, **13**, 13. DOI: 10.1186/1471-2288-13-13.

Horowitz, M., Gale, R., Sondel, P., et al. (1990) Graft-versus-leukemia reactions after bone marrow transplantation. *Blood*, **75**, 555–562. DOI: 10.1182/blood.V75.3.555.555.

Horowitz, M. M. (2008) The role of registries in facilitating clinical research in BMT: Examples from the Center for International Blood and Marrow Transplant Research. *Bone Marrow Transplantation*, **42**, S1–S2. DOI: 10.1038/bmt.2008.101.

Huang, A., Chen, Q., Fei, Y., et al. (2021) Dynamic prediction of relapse in patients with acute leukemias after allogeneic transplantation: Joint model for minimal residual disease. *International Journal of Laboratory Hematology*, **43**, 84–92. DOI: 10.1111/ijlh.13328.

Hughes, R. A., Heron, J., Sterne, J. A. C., et al. (2019) Accounting for missing data in statistical analyses: Multiple imputation is not always the answer. *International Journal of Epidemiology*, **48**, 1294–1304. DOI: 10.1093/ije/dyz032.

Inoue, Y., Nakano N., Fuji S., et al. (2021) Impact of conditioning intensity and regimen on transplant outcomes in patients with adult T-cell leukemia-lymphoma. *Bone Marrow Transplantation*, **56**, 2964–2974. DOI: 10.1038/s41409-021-01445-0.

Ito, A., Kitano, S., Tajima, K., et al. (2020) Impact of low-dose anti-thymocyte globulin on immune reconstitution after allogeneic hematopoietic cell transplantation. *International Journal of Hematology*, **111**, 120–130. DOI: 10.1007/s12185-019-02756-1.

Jeong, J.-H. and Fine, J. (2006) Direct parametric inference for the cumulative incidence function. *Journal of the Royal Statistical Society: Series C (Applied Statistics)*, **55**, 187–200. DOI: 10.1111/j.1467-9876.2006.00532.x.

Kalbfleisch, J. D. and Prentice, R. L. (2011) *The Statistical Analysis of Failure Time Data*. Wiley series in probability and statistics.

Kantidakis, G., Putter, H., Litière, S., et al. (2023) Statistical models versus machine learning for competing risks: Development and validation of prognostic models. *BMC Medical Research Methodology*, **23**, 51. DOI: 10.1186/s12874-023-01866-z.

Kaplan, E. L. and Meier, P. (1958) Nonparametric Estimation from Incomplete Observations. *Journal of the American Statistical Association*, **53**, 457–481. DOI: 10.2307/2281868.

Keogh, R. H. and Morris, T. P. (2018) Multiple imputation in Cox regression when there are time-varying effects of covariates. *Statistics in Medicine*, **37**, 3661–3678. DOI: 10.1002/sim.7842.

Kipourou, D.-K., Charvat, H., Rachet, B., et al. (2019) Estimation of the adjusted cause-specific cumulative probability using flexible regression models for the cause-specific hazards. *Statistics in Medicine*, **38**, 3896–3910. DOI: 10.1002/sim.8209.

Klein, J. P. and Andersen, P. K. (2005) Regression Modeling of Competing Risks Data Based on Pseudovalues of the Cumulative Incidence Function. *Biometrics*, **61**, 223–229. DOI: 10.1111/j.0006-341X.2005.031209.x.

Klein, J. P. and Moeschberger, M. L. (2003) *Survival Analysis: Techniques for Censored and Truncated Data (2nd Edition)*.

Kleinke, K. (2017) Multiple Imputation Under Violated Distributional Assumptions: A Systematic Evaluation of the Assumed Robustness of Predictive Mean Matching. *Journal of Educational and Behavioral Statistics*, **42**, 371–404. DOI: 10.3102/1076998616687084.

Koster, E. A. S., von dem Borne, P. A., van Balen, P., et al. (2023) Competitive Repopulation and Allo-Immunologic Pressure Determine Chimerism Kinetics after T Cell-Depleted Allogeneic Stem Cell Transplantation and Donor Lympho-

cyte Infusion. *Transplantation and Cellular Therapy*, **29**, 268.e1–268.e10. DOI: 10.1016/j.jtct.2022.12.022.

Krishnamurthy, P., Potter, V. T., Barber, L. D., et al. (2013) Outcome of Donor Lymphocyte Infusion after T Cell–depleted Allogeneic Hematopoietic Stem Cell Transplantation for Acute Myelogenous Leukemia and Myelodysplastic Syndromes. *Biology of Blood and Marrow Transplantation*, **19**, 562–568. DOI: 10.1016/j.bbmt.2012.12.013.

Kröger, N., Bacigalupo, A., Barbui, T., et al. (2024) Indication and management of allogeneic haematopoietic stem-cell transplantation in myelofibrosis: Updated recommendations by the EBMT/ELN International Working Group. *The Lancet Haematology*, **11**, e62–e74. DOI: 10.1016/S2352-3026(23)00305-8.

Lambert, P. C., Wilkes, S. R. and Crowther, M. J. (2017) Flexible parametric modelling of the cause-specific cumulative incidence function. *Statistics in Medicine*, **36**, 1429–1446. DOI: 10.1002/sim.7208.

Landau, W. M. (2021) The targets R package: A dynamic Make-like function-oriented pipeline toolkit for reproducibility and high-performance computing. *Journal of Open Source Software*, **6**, 2959. DOI: 10.21105/joss.02959.

Latouche, A., Boisson, V., Chevret, S., et al. (2007) Misspecified regression model for the subdistribution hazard of a competing risk. *Statistics in Medicine*, **26**, 965–974. DOI: 10.1002/sim.2600.

Latouche, A., Allignol, A., Beyersmann, J., et al. (2013) A competing risks analysis should report results on all cause-specific hazards and cumulative incidence functions. *Journal of Clinical Epidemiology*, **66**, 648–653. DOI: 10.1016/j.jclinepi.2012.09.017.

Lau, B. and Lesko, C. (2018) Missingness in the Setting of Competing Risks: From Missing Values to Missing Potential Outcomes. *Current Epidemiology Reports*, **5**, 153–159. DOI: 10.1007/s40471-018-0142-3.

Lee, K. J. and Carlin, J. B. (2017) Multiple imputation in the presence of non-normal data. *Statistics in Medicine*, **36**, 606–617. DOI: 10.1002/sim.7173.

Lee, K. J., Tilling, K. M., Cornish, R. P., et al. (2021) Framework for the treatment and reporting of missing data in observational studies: The Treatment And Reporting of Missing data in Observational Studies framework. *Journal of Clinical Epidemiology*, **134**, 79–88. DOI: 10.1016/j.jclinepi.2021.01.008.

Lee, K. J., Carlin, J. B., Simpson, J. A., et al. (2023) Assumptions and analysis planning in studies with missing data in multiple variables: Moving beyond the

MCAR/MAR/MNAR classification. *International Journal of Epidemiology*, **52**, 1268–1275. DOI: 10.1093/ije/dyad008.

Lennon, H., Sperrin, M., Badrick, E., et al. (2016) The Obesity Paradox in Cancer: A Review. *Current Oncology Reports*, **18**, 56. DOI: 10.1007/s11912-016-0539-4.

Lewalle, P., Triffet, A., Delforge, A., et al. (2003) Donor lymphocyte infusions in adult haploidentical transplant: A dose finding study. *Bone Marrow Transplantation*, **31**, 39–44. DOI: 10.1038/sj.bmt.1703779.

Little, R. J. (2021) Missing Data Assumptions. *Annual Review of Statistics and Its Application*, **8**, 89–107. DOI: 10.1146/annurev-statistics-040720-031104.

Little, R. J. (2024) Missing Data Analysis. *Annual Review of Clinical Psychology*, **20**, 149–173. DOI: 10.1146/annurev-clinpsy-080822-051727.

Little, R. J. and Rubin, D. B. (2019) *Statistical Analysis with Missing Data*.

Little, R. J. A. (1995) Modeling the Drop-Out Mechanism in Repeated-Measures Studies. *Journal of the American Statistical Association*, **90**, 1112–1121. DOI: 10.1080/01621459.1995.10476615.

Little, R. J. A. and Rubin, D. B. (1987) Statistical analysis with missing data. *New York: Wiley*.

Liu, J., Gelman, A., Hill, J., et al. (2014) On the stationary distribution of iterative imputations. *Biometrika*, **101**, 155–173. DOI: 10.1093/biomet/ast044.

Lohmann, A., Astivia, O. L. O., Morris, T. P., et al. (2022) It's time! Ten reasons to start replicating simulation studies. *Frontiers in Epidemiology*, **2**. DOI: 10.3389/fepid.2022.973470.

Madley-Dowd, P., Hughes, R., Tilling, K., et al. (2019) The proportion of missing data should not be used to guide decisions on multiple imputation. *Journal of Clinical Epidemiology*, **110**, 63–73. DOI: 10.1016/j.jclinepi.2019.02.016.

Mainzer, R. M., Moreno-Betancur, M., Nguyen, C. D., et al. (2024) Gaps in the usage and reporting of multiple imputation for incomplete data: Findings from a scoping review of observational studies addressing causal questions. *BMC Medical Research Methodology*, **24**, 193. DOI: 10.1186/s12874-024-02302-6.

Mao, L. and Lin, D. Y. (2017) Efficient Estimation of Semiparametric Transformation Models for the Cumulative Incidence of Competing Risks. *Journal of the*

Royal Statistical Society Series B: Statistical Methodology, **79**, 573–587. DOI: 10.1111/rssb.12177.

Mariano, L., Zhang, B. M., Osoegawa, K., et al. (2019) Assessment by Extended-Coverage Next-Generation Sequencing Typing of DPA1 and DPB1 Mismatches in Siblings Matching at HLA-A, -B, -C, -DRB1, and -DQ Loci. *Biology of Blood and Marrow Transplantation*, **25**, 2507–2509. DOI: 10.1016/j.bbmt.2019.07.033.

Marini, M. M., Olsen, A. R. and Rubin, D. B. (1980) Maximum-Likelihood Estimation in Panel Studies with Missing Data. *Sociological Methodology*, **11**, 314–357. DOI: 10.2307/270868.

Marshall, A., Altman, D. G., Holder, R. L., et al. (2009) Combining estimates of interest in prognostic modelling studies after multiple imputation: Current practice and guidelines. *BMC Medical Research Methodology*, **9**, 57. DOI: 10.1186/1471-2288-9-57.

Marshall, A., Altman, D. G. and Holder, R. L. (2010) Comparison of imputation methods for handling missing covariate data when fitting a Cox proportional hazards model: A resampling study. *BMC Medical Research Methodology*, **10**, 112. DOI: 10.1186/1471-2288-10-112.

Martinussen, T. and Stensrud, M. J. (2023) Estimation of separable direct and indirect effects in continuous time. *Biometrics*, **79**, 127–139. DOI: 10.1111/biom.13559.

McCullagh, P. and Nelder, J. A. (1989) *Generalized Linear Models, Second Edition*. Chapman and Hall/CRC monographs on statistics and applied probability series.

McCurdy, S. R., Radojcic, V., Tsai, H.-L., et al. (2022) Signatures of GVHD and relapse after posttransplant cyclophosphamide revealed by immune profiling and machine learning. *Blood*, **139**, 608–623. DOI: 10.1182/blood.2021013054.

Meng, X.-L. (1994) Multiple-Imputation Inferences with Uncongenial Sources of Input. *Statistical Science*, **9**, 538–558. DOI: 10.1214/ss/1177010269.

Mertens, B. J. A., Banzato, E. and de Wreede, L. C. (2020) Construction and assessment of prediction rules for binary outcome in the presence of missing predictor data using multiple imputation and cross-validation: Methodological approach and data-based evaluation. *Biometrical Journal*, **62**, 724–741. DOI: 10.1002/bimj.201800289.

Minculescu, L., Marquart, H. V., Friis, L. S., et al. (2016) Early Natural Killer Cell Reconstitution Predicts Overall Survival in T Cell–Replete Allogeneic Hematopoietic Stem Cell Transplantation. *Biology of Blood and Marrow Transplantation*, **22**, 2187–2193. DOI: 10.1016/j.bbmt.2016.09.006.

Molenberghs, G. and Fitzmaurice, G. (2008) Incomplete data: Introduction and overview. In *Longitudinal Data Analysis*.

Molenberghs, G., Fitzmaurice, G., Kenward, M. G., et al. (2014) *Handbook of Missing Data Methodology*.

Monterrubio-Gómez, K., Constantine-Cooke, N. and Vallejos, C. A. (2024) A review on statistical and machine learning competing risks methods. *Biometrical Journal*, **66**, 2300060. DOI: 10.1002/bimj.202300060.

Morisot, A., Bessaoud, F., Landais, P., et al. (2015) Prostate cancer: Net survival and cause-specific survival rates after multiple imputation. *BMC Medical Research Methodology*, **15**, 54. DOI: 10.1186/s12874-015-0048-4.

Morris, T. P., White, I. R. and Crowther, M. J. (2019) Using simulation studies to evaluate statistical methods. *Statistics in Medicine*, **38**, 2074–2102. DOI: 10.1002/sim.8086.

Mozumder, S. I., Rutherford, M. and Lambert, P. (2018) Direct likelihood inference on the cause-specific cumulative incidence function: A flexible parametric regression modelling approach. *Statistics in Medicine*, **37**, 82–97. DOI: 10.1002/sim.7498.

Murray, J. S. (2018) Multiple Imputation: A Review of Practical and Theoretical Findings. *Statistical Science*, **33**, 142–159. DOI: 10.1214/18-STS644.

Nikiforow, S., Kim, H. T., Daley, H., et al. (2016) A phase I study of CD25/regulatory T-cell-depleted donor lymphocyte infusion for relapse after allogeneic stem cell transplantation. *Haematologica*, **101**, 1251–1259. DOI: 10.3324/haematol.2015.141176.

Nowok, B., Raab, G. M. and Dibben, C. (2016) Synthpop: Bespoke Creation of Synthetic Data in R. *Journal of Statistical Software*, **74**, 1–26. DOI: 10.18637/jss.v074.i11.

Ozenne, B., Sørensen, A. L., Scheike, T., et al. (2017) riskRegression: Predicting the risk of an event using cox regression models. *The R Journal*, **9**, 440–460. DOI: 10.32614/RJ-2017-062.

Papageorgiou, G. and Rizopoulos, D. (2021) An alternative characterization of MAR in shared parameter models for incomplete longitudinal data and its utilization for sensitivity analysis. *Statistical Modelling*, **21**, 95–114. DOI: 10.1177/1471082X20927114.

Papageorgiou, G., Mauff, K., Tomer, A., et al. (2019) An Overview of Joint Modeling of Time-to-Event and Longitudinal Outcomes. *Annual Review of Statistics and Its Application*, **6**, 223–240. DOI: 10.1146/annurev-statistics-030718-105048.

Passamonti, F., Cervantes, F., Vannucchi, A. M., et al. (2010) A dynamic prognostic model to predict survival in primary myelofibrosis: A study by the IWG-MRT (International Working Group for Myeloproliferative Neoplasms Research and Treatment). *Blood*, **115**, 1703–1708. DOI: 10.1182/blood-2009-09-245837.

Paz, D. L., Riou J., Verger E., et al. (2021) Genomic analysis of primary and secondary myelofibrosis redefines the prognostic impact of ASXL1 mutations: A FIM study. *Blood Advances*, **5**, 1442–1451. DOI: 10.1182/bloodadvances.2020003444.

Penack, O., Fischer, L., Stroux, A., et al. (2008) Serotherapy with thymoglobulin and alemtuzumab differentially influences frequency and function of natural killer cells after allogeneic stem cell transplantation. *Bone Marrow Transplantation*, **41**, 377–383. DOI: 10.1038/sj.bmt.1705911.

Pinheiro, J., Bates, D. and R Core Team (2023) *Nlme: Linear and Nonlinear Mixed Effects Models*. Manual.

Polverelli, N., Tura, P., Battipaglia, G., et al. (2020) Multidimensional geriatric assessment for elderly hematological patients (≥60 years) submitted to allogeneic stem cell transplantation. A French–Italian 10-year experience on 228 patients. *Bone Marrow Transplantation*, **55**, 2224–2233. DOI: 10.1038/s41409-020-0934-1.

Polverelli, N., Mauff, K., Kröger, N., et al. (2021) Impact of spleen size and splenectomy on outcomes of allogeneic hematopoietic cell transplantation for myelofibrosis: A retrospective analysis by the chronic malignancies working party on behalf of European society for blood and marrow transplantation (EBMT). *American Journal of Hematology*, **96**, 69–79. DOI: 10.1002/ajh.26020.

Polverelli, N., Hernández-Boluda, J. C., Czerw, T., et al. (2023) Splenomegaly in patients with primary or secondary myelofibrosis who are candidates for allogeneic hematopoietic cell transplantation: A Position Paper on behalf of the Chronic Malignancies Working Party of the EBMT. *The Lancet Haematology*, **10**, e59–e70. DOI: 10.1016/S2352-3026(22)00330-1.

Polverelli, N., Bonneville, E. F., de Wreede, L. C., et al. (2024) Impact of comorbidities and body mass index on the outcomes of allogeneic hematopoietic cell transplantation in myelofibrosis: A study on behalf of the Chronic Malignancies Working Party of EBMT. *American journal of hematology*, **99**, 993—996. DOI: 10.1002/ajh.27262.

Poythress, J. c., Lee, M. Y. and Young, J. (2020) Planning and analyzing clinical trials with competing risks: Recommendations for choosing appropriate statistical methodology. *Pharmaceutical Statistics*, **19**, 4–21. DOI: 10.1002/pst.1966.

Prentice, R. L., Kalbfleisch, J. D., Peterson, A. V., et al. (1978) The Analysis of Failure Times in the Presence of Competing Risks. *Biometrics*, **34**, 541–554. DOI: 10.2307/2530374.

Putter, H., Fiocco, M. and Geskus, R. B. (2007) Tutorial in biostatistics: Competing risks and multi-state models. *Statistics in Medicine*, **26**, 2389–2430. DOI: 10.1002/sim.2712.

Putter, H., Schumacher, M. and van Houwelingen, H. C. (2020) On the relation between the cause-specific hazard and the subdistribution rate for competing risks data: The Fine–Gray model revisited. *Biometrical Journal*, **62**, 790–807. DOI: 10.1002/bimj.201800274.

Qi, L., Wang, Y.-F. and He, Y. (2010) A comparison of multiple imputation and fully augmented weighted estimators for Cox regression with missing covariates. *Statistics in Medicine*, **29**, 2592–2604. DOI: 10.1002/sim.4016.

Quartagno, M. and Carpenter, J. R. (2019) Multiple imputation for discrete data: Evaluation of the joint latent normal model. *Biometrical Journal*, **61**, 1003–1019. DOI: 10.1002/bimj.201800222.

R Core Team (2020) *R: A Language and Environment for Statistical Computing*. Manual.

R Core Team (2023) *R: A Language and Environment for Statistical Computing*. Manual.

R Core Team (2024) *R: A Language and Environment for Statistical Computing*. Manual.

Rathouz, P. J. (2007) Identifiability assumptions for missing covariate data in failure time regression models. *Biostatistics*, **8**, 345–356. DOI: 10.1093/biostatistics/kxl014.

Resche-Rigon, M., White, I. and Chevret, S. (2012) Imputing missing covariate values in presence of competing risk. In: *International Society for Clinical Biostatistics Conference*, Bergen, Norway, 19-23 August 2012, P22.10, August 2012.

Rizopoulos, D. (2010) JM: An R package for the joint modelling of longitudinal and time-to-event data. *Journal of Statistical Software*, **35**, 1–33. DOI: 10.18637/jss.v035.i09.

Rizopoulos, D. (2012) *Joint Models for Longitudinal and Time-to-Event Data*. 1st edition.

Rizopoulos, D., Papageorgiou, G. and Miranda Afonso, P. (2023) *JMbayes2: Extended Joint Models for Longitudinal and Time-to-Event Data*. Manual.

Rouanet, A., Helmer, C., Dartigues, J.-F., et al. (2019) Interpretation of mixed models

and marginal models with cohort attrition due to death and drop-out. *Statistical Methods in Medical Research*, **28**, 343–356. DOI: 10.1177/0962280217723675.

Ruan, P. K. and Gray, R. J. (2008) Analyses of cumulative incidence functions via non-parametric multiple imputation. *Statistics in Medicine*, **27**, 5709–5724. DOI: 10.1002/sim.3402.

Rubin, D. (1987) *Multiple Imputation for Nonresponse in Surveys*. DOI: 10.1002/9780470316696.

Rubin, D. B. (1976) Inference and missing data. *Biometrika*, **63**, 581–592. DOI: 10.1093/biomet/63.3.581.

Rücker, G. and Schwarzer, G. (2014) Presenting simulation results in a nested loop plot. *BMC Medical Research Methodology*, **14**, 129. DOI: 10.1186/1471-2288-14-129.

Saadati, M., Beyersmann, J., Kopp-Schneider, A., et al. (2018) Prediction accuracy and variable selection for penalized cause-specific hazards models. *Biometrical Journal*, **60**, 288–306. DOI: 10.1002/bimj.201600242.

Saccardi, R., Putter, H., Eikema, D.-J., et al. (2023) Benchmarking of survival outcomes following Haematopoietic Stem Cell Transplantation (HSCT): An update of the ongoing project of the European Society for Blood and Marrow Transplantation (EBMT) and Joint Accreditation Committee of ISCT and EBMT (JACIE). *Bone Marrow Transplantation*, **58**, 659–666. DOI: 10.1038/s41409-023-01924-6.

Salzmann-Manrique, E., Bremm, M., Huenecke, S., et al. (2018) Joint Modeling of Immune Reconstitution Post Haploidentical Stem Cell Transplantation in Pediatric Patients With Acute Leukemia Comparing CD34+-Selected to CD3/CD19-Depleted Grafts in a Retrospective Multicenter Study. *Frontiers in Immunology*, **9**. DOI: 10.3389/fimmu.2018.01841.

Schetelig, J., Wreede, L. C. de, Gelder, M. van, et al. (2019) Late treatment-related mortality versus competing causes of death after allogeneic transplantation for myelodysplastic syndromes and secondary acute myeloid leukemia. *Leukemia*, **33**, 686–695. DOI: 10.1038/s41375-018-0302-y.

Schluchter, M. D. and Jackson, K. L. (1989) Log-Linear Analysis of Censored Survival Data with Partially Observed Covariates. *Journal of the American Statistical Association*, **84**, 42–52. DOI: 10.1080/01621459.1989.10478737.

Schmaelter, A.-K., Waidhauser, J., Kaiser, D., et al. (2021) Alterations of Peripheral Blood T Cell Subsets following Donor Lymphocyte Infusion in Patients after Allogeneic

Stem Cell Transplantation. *Hemato*, **2**, 692–702. DOI: 10.3390/hemato2040046.

Schultze-Florey, C. R., Kuhlmann, L., Raha, S., et al. (2021) Clonal expansion of CD8+ T cells reflects graft-versus-leukemia activity and precedes durable remission following DLI. *Blood Advances*, **5**, 4485–4499. DOI: 10.1182/bloodadvances.2020004073.

Seaman, S., Galati, J., Jackson, D., et al. (2013) What Is Meant by "Missing at Random"? *Statistical Science*, **28**, 257–268. DOI: 10.1214/13-STS415.

Shah, A. D., Bartlett, J. W., Carpenter, J., et al. (2014) Comparison of Random Forest and Parametric Imputation Models for Imputing Missing Data Using MICE: A CALIBER Study. *American Journal of Epidemiology*, **179**, 764–774. DOI: 10.1093/aje/kwt312.

Sharma, A., Huang S., Li Y., et al. (2021) Outcomes of pediatric patients with therapy-related myeloid neoplasms. *Bone Marrow Transplantation*, **56**, 2997–3007. DOI: 10.1038/s41409-021-01448-x.

Shaw, B. E., Gooley, T. A., Malkki, M., et al. (2007) The importance of HLA-DPB1 in unrelated donor hematopoietic cell transplantation. *Blood*, **110**, 4560–4566. DOI: 10.1182/blood-2007-06-095265.

Shi, H., Cheng, Y. and Jeong, J.-H. (2013) Constrained parametric model for simultaneous inference of two cumulative incidence functions. *Biometrical Journal*, **55**, 82–96. DOI: 10.1002/bimj.201200011.

Shimoni, A., Labopin, M., Savani, B., et al. (2016) Long-term survival and late events after allogeneic stem cell transplantation from HLA-matched siblings for acute myeloid leukemia with myeloablative compared to reduced-intensity conditioning: A report on behalf of the acute leukemia working party of European group for blood and marrow transplantation. *Journal of Hematology & Oncology*, **9**, 118. DOI: 10.1186/s13045-016-0347-1.

Simonetta, F., Alvarez, M. and Negrin, R. S. (2017) Natural Killer Cells in Graft-versus-Host-Disease after Allogeneic Hematopoietic Cell Transplantation. *Frontiers in Immunology*, **8**. DOI: 10.3389/fimmu.2017.00465.

Snowden, J. A., Saccardi, R., Orchard, K., et al. (2020) Benchmarking of survival outcomes following haematopoietic stem cell transplantation: A review of existing processes and the introduction of an international system from the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE). *Bone Marrow Transplantation*, **55**, 681–694. DOI: 10.1038/s41409-019-0718-7.

Sorror, M. L., Maris, M. B., Storb, R., et al. (2005) Hematopoietic cell transplantation (HCT)-specific comorbidity index: A new tool for risk assessment before allogeneic HCT. *Blood*, **106**, 2912–2919. DOI: 10.1182/blood-2005-05-2004.

Stern, L., McGuire, H. M., Avdic, S., et al. (2022) Immunoprofiling reveals cell subsets associated with the trajectory of cytomegalovirus reactivation post stem cell transplantation. *Nature Communications*, **13**, 2603. DOI: 10.1038/s41467-022-29943-9.

Sterne, J. A., Hernán, M. A., Reeves, B. C., et al. (2016) ROBINS-I: A tool for assessing risk of bias in non-randomised studies of interventions. *BMJ*, **355**, i4919. DOI: 10.1136/bmj.i4919.

Sterne, J. A. C., White, I. R., Carlin, J. B., et al. (2009) Multiple imputation for missing data in epidemiological and clinical research: Potential and pitfalls. *BMJ*, **338**, b2393. DOI: 10.1136/bmj.b2393.

Sullivan, T. R., Yelland, L. N., Lee, K. J., et al. (2017) Treatment of missing data in follow-up studies of randomised controlled trials: A systematic review of the literature. *Clinical Trials*, **14**, 387–395. DOI: 10.1177/1740774517703319.

Sullivan, T. R., White, I. R., Salter, A. B., et al. (2018) Should multiple imputation be the method of choice for handling missing data in randomized trials? *Statistical Methods in Medical Research*, **27**, 2610–2626. DOI: 10.1177/0962280216683570.

Sweeting, M. J. and Thompson, S. G. (2011) Joint modelling of longitudinal and time-to-event data with application to predicting abdominal aortic aneurysm growth and rupture. *Biometrical Journal*, **53**, 750–763. DOI: 10.1002/bimj.201100052.

Tang, X., Alatrash, G., Ning, J., et al. (2014) Increasing Chimerism after Allogeneic Stem Cell Transplantation Is Associated with Longer Survival Time. *Biology of Blood and Marrow Transplantation*, **20**, 1139–1144. DOI: 10.1016/j.bbmt.2014.04.003.

Taylor, J. M. G., Murray, S. and Hsu, C.-H. (2002) Survival estimation and testing via multiple imputation. *Statistics & Probability Letters*, **58**, 221–232. DOI: 10.1016/S0167-7152(02)00030-5.

Therneau, T. M. (2023) *A Package for Survival Analysis in R*. Manual.

Tompsett, D. M., Leacy, F., Moreno-Betancur, M., et al. (2018) On the use of the not-at-random fully conditional specification (NARFCS) procedure in practice. *Statistics in Medicine*, **37**, 2338–2353. DOI: 10.1002/sim.7643.

Toor, A. A., Sabo, R. T., Roberts, C. H., et al. (2015) Dynamical System Modeling of

Immune Reconstitution after Allogeneic Stem Cell Transplantation Identifies Patients at Risk for Adverse Outcomes. *Biology of Blood and Marrow Transplantation*, **21**, 1237–1245. DOI: 10.1016/j.bbmt.2015.03.011.

Uhl, F. M., Chen, S., O'Sullivan, D., et al. (2020) Metabolic reprogramming of donor T cells enhances graft-versus-leukemia effects in mice and humans. *Science Translational Medicine*, **12**, eabb8969. DOI: 10.1126/scitranslmed.abb8969.

van Buuren, S. (2018) *Flexible Imputation of Missing Data, Second Edition*.

van Buuren, S. and Groothuis-Oudshoorn, K. (2011) Mice: Multivariate Imputation by Chained Equations in R. *Journal of Statistical Software*, **45**, 1–67. DOI: 10.18637/jss.v045.i03.

van Buuren, S., Oudshoorn, K. and TNO Preventie en Gezondheid (1999) *Flexible Multivariate Imputation by MICE*. January.

van Buuren, S., Brand, J. P. L., Groothuis-Oudshoorn, C. G. M., et al. (2006) Fully conditional specification in multivariate imputation. *Journal of Statistical Computation and Simulation*, **76**, 1049–1064. DOI: 10.1080/10629360600810434.

van der Pas, S., Nelissen, R. and Fiocco, M. (2018) Different competing risks models for different questions may give similar results in arthroplasty registers in the presence of few events. *Acta Orthopaedica*, **89**, 145–151. DOI: 10.1080/17453674.2018.1427314.

van Houwelingen, H. and Putter, H. (2012) *Dynamic Prediction in Clinical Survival Analysis*. DOI: 10.1201/b11311.

Vandenbroucke, J. P., Elm, E. von, Altman, D. G., et al. (2007) Strengthening the Reporting of Observational Studies in Epidemiology (STROBE): Explanation and Elaboration. *PLOS Medicine*, **4**, e297. DOI: 10.1371/journal.pmed.0040297.

Vicente-Saez, R. and Martinez-Fuentes, C. (2018) Open Science now: A systematic literature review for an integrated definition. *Journal of Business Research*, **88**, 428–436. DOI: 10.1016/j.jbusres.2017.12.043.

von dem Borne, P. A., Starrenburg, C. W. J. I., Halkes, S. J. M., et al. (2009) Reduced-intensity conditioning allogeneic stem cell transplantation with donor T-cell depletion using alemtuzumab added to the graft ('Campath in the bag'). *Current Opinion in Oncology*, **21 Suppl 1**, S27–29. DOI: 10.1097/01.cco.0000357472.76337.0e.

von Hippel, P. T. (2020) How Many Imputations Do You Need? A Two-stage Calculation Using a Quadratic Rule. *Sociological Methods & Research*, **49**, 699–718. DOI:

10.1177/0049124117747303.

White, I. R. and Carlin, J. B. (2010) Bias and efficiency of multiple imputation compared with complete-case analysis for missing covariate values. *Statistics in Medicine*, **29**, 2920–2931. DOI: 10.1002/sim.3944.

White, I. R. and Royston, P. (2009) Imputing missing covariate values for the Cox model. *Statistics in Medicine*, **28**, 1982–1998. DOI: 10.1002/sim.3618.

White, I. R. and Thompson, S. G. (2005) Adjusting for partially missing baseline measurements in randomized trials. *Statistics in Medicine*, **24**, 993–1007. DOI: 10.1002/sim.1981.

White, I. R., Pandis, N. and Pham, T. M. (2022) Missing data, part 7. Pitfalls in doing multiple imputation. *American Journal of Orthodontics and Dentofacial Orthopedics*, **162**, 975–977. DOI: 10.1016/j.ajodo.2022.08.013.

Wolbers, M., Koller, M. T., Witteman, J. C. M., et al. (2009) Prognostic Models With Competing Risks: Methods and Application to Coronary Risk Prediction. *Epidemiology*, **20**, 555–561. DOI: 10.1097/EDE.0b013e3181a39056.

Wood, A. M., Royston, P. and White, I. R. (2015) The estimation and use of predictions for the assessment of model performance using large samples with multiply imputed data. *Biometrical Journal*, **57**, 614–632. DOI: 10.1002/bimj.201400004.

Yanir, A., Schulz, A., Lawitschka, A., et al. (2022) Immune Reconstitution After Allogeneic Haematopoietic Cell Transplantation: From Observational Studies to Targeted Interventions. *Frontiers in Pediatrics*, **9**. DOI: 10.3389/fped.2021.786017.

Young, J. G., Stensrud, M. J., Tchetgen Tchetgen, E. J., et al. (2020) A causal framework for classical statistical estimands in failure-time settings with competing events. *Statistics in Medicine*, **39**, 1199–1236. DOI: 10.1002/sim.8471.

Yun, H. D. and Waller, E. K. (2013) Finding the Sweet Spot for Donor Lymphocyte Infusions. *Biology of Blood and Marrow Transplantation*, **19**, 507–508. DOI: 10.1016/j.bbmt.2013.02.005.

Zhu, J. and Raghunathan, T. E. (2015) Convergence Properties of a Sequential Regression Multiple Imputation Algorithm. *Journal of the American Statistical Association*, **110**, 1112–1124. DOI: 10.1080/01621459.2014.948117.