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The European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) Guide on Methodological Standards in Pharmacoepidemiology

(Revision 9)

References

The hyperlinks in the document were last accessed online in June 2021.

Chapter 1 – Introduction

A. Hernán, M., & James, M. R. (2020). Causal Inference: What If. Chapman & Hall.

Ali A, Hartzema A. (2018). Post-Authorization Safety Studies of Medicinal Products. The PASS Book (1st ed.). Academic Press.

Altman, D. (2020). Practical Statistics for Medical Research. 2nd: Chapman & Hall.

Andrews AB, Moore N. (2014). Mann's Pharmacovigilance (3rd ed.). Wiley Blackwell.

Cave, A., Kurz, X., & Arlett, P. (2019). Real-World Data for Regulatory Decision Making: Challenges and Possible Solutions for Europe. Clinical Pharmacology & Therapeutics, 106(1), 36–39. https://doi.org/10.1002/cpt.1426

Directive 2001/83/EC of the European Parliament and of the Council of 6 November 2001. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=CONSLEG:2001L0083:20070126:en:PDF

Elseviers M, Wettermark B, Almarsdóttir AB, et al. (2016). Drug Utilization Research: Methods and Applications. Wiley Blackwell.

FDA's Real-World Evidence website. FDA. https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Hartzema AG, Tilson HH, Chan KA. (2008). Pharmacoepidemiology and Therapeutic Risk Management (3rd ed.). Harvey Whitney Books Company.

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Hoffmann, W., Latza, U., Baumeister, S. E., Brünger, M., Buttmann-Schweiger, N., Hardt, J., et al. (2019). Guidelines and recommendations for ensuring Good Epidemiological Practice (GEP): a guideline developed by the German Society for Epidemiology. European Journal of Epidemiology, 34(3), 301–317. https://doi.org/10.1007/s10654-019-00500-x

IEA Good Epidemiology Practices (GEP). (2007). IEA Publications. https://ieaweb.org/IEAWeb/Content/IEA_Publications.aspx

ISPE Guidelines for Good Pharmacoepidemiology Practices (GPP). (n.d.). International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Miettinen, O. S. (1985). Theoretical Epidemiology: Principles of Occurrence Research in Medicines. Wiley.

Parfrey PS, Barret BJ. (2015). Clinical epidemiology: practice and methods (2nd ed.). Human Press.

Porta M, Last JM, Greenland. (2014). A Dictionary of Epidemiology (6th ed.). Oxford University Press.

Rothman K, Greenland S, Lash T. (2008). Modern Epidemiology (3th ed.). Lippincott Williams & Wilkins.

Scientific guidance on post-authorisation efficacy studies - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/scientific-guidance-post-authorisation-efficacy-studies-first-version_en.pdf

Strom B, Kimmel SE, Hennessy S. (2019). Pharmacoepidemiology (6th ed.). Wiley.

Woodward, M. (2014). Epidemiology: Study Design and Data Analysis (3rd ed.). Chapman & Hall.

Chapter 2 - Formulating the research question and objectives

Brown, P., Brunnhuber, K., Chalkidou, K., Chalmers, I., Clarke, M., Fenton, M., et al. (2006). How to formulate research recommendations. BMJ : British Medical Journal, 333(7572), 804–806. https://doi.org/10.1136/bmj.38987.492014.94

Criteria to select and prioritize health technologies for additional evidence generation - EUnetHTA. https://eunethta.eu/wp-content/uploads/2018/01/Selection-prioritisation-criteria-1.pdf. Accessed 4 May 2021

European Medicines Agency. European Medicines Agency. https://www.ema.europa.eu/en

Good pharmacovigilance practices. (2018, September 17). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

ISPE Guidelines for Good Pharmacoepidemiology Practices (GPP) - International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Kloda, L., & Bartlett, J. C. (2013). Formulating Answerable Questions: Question Negotiation in Evidence-based Practice. Journal of the Canadian Health Libraries Association / Journal de l'Association des bibliothèques de la santé du Canada, 34(2), 55–60. https://doi.org/10.5596/c13-019

Lipowski, E. E. (2008). Developing great research questions. American Journal of Health-System Pharmacy, 65(17), 1667–1670. https://doi.org/10.2146/ajhp070276

Owen Doody, M. E. B. (2014). Setting a research question, aim and objective. https://doi.org/10.7748/nr.23.4.19.s5

Position paper on how to best formulate research recommendations - EUnetHTA. https://eunethta.eu/wp-content/uploads/2018/01/eunethta_position_paper_on_research_recommendations_0-1.pdf

Ritchey, M. E., & Girman, C. J. (2020). Evaluating the Feasibility of Electronic Health Records and Claims Data Sources for Specific Research Purposes. Therapeutic Innovation & Regulatory Science, 54(6), 1296–1302. https://doi.org/10.1007/s43441-020-00139-x

Rivera, D. R., Gokhale, M. N., Reynolds, M. W., Andrews, E. B., Chun, D., Haynes, K., et al. (2020). Linking electronic health data in pharmacoepidemiology: Appropriateness and feasibility. Pharmacoepidemiology and Drug Safety, 29(1), 18–29. https://doi.org/10.1002/pds.4918

Thabane, L., Thomas, T., Ye, C., & Paul, J. (2009). Posing the research question: not so simple. Canadian Journal of Anesthesia/Journal canadien d'anesthésie, 56(1), 71–79. https://doi.org/10.1007/s12630-008-9007-4

There's a S.M.A.R.T. way to write management's goals and objectives. (n.d.). https://community.mis.temple.edu/mis0855002fall2015/files/2015/10/S.M.A.R.T-Way-Management-Review.pdf

Tully, M. P. (2014). Research: Articulating Questions, Generating Hypotheses, and Choosing Study Designs. Canadian Journal of Hospital Pharmacy, 67(1). https://doi.org/10.4212/cjhp.v67i1.1320

Willame, C., Baril, L., van den Bosch, J., Ferreira, G. L. C., Williams, R., Rosillon, D., & Cohet, C. (2016). Importance of feasibility assessments before implementing non-interventional pharmacoepidemiologic studies of vaccines: lessons learned and recommendations for future studies. Pharmacoepidemiology and Drug Safety, 25(12), 1397–1406. https://doi.org/10.1002/pds.4081

Chapter 3 – Development of the study protocol

Agency for Healthcare Research and Quality (AHRQ). https://www.ahrq.gov/. Accessed 27 May 2021

Best Practices for Conducting and Reporting Pharmacoepidemiologic Safety Studies Using Electronic Healthcare Data Sets. FDA. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/best-practices-conducting-and-reporting-pharmacoepidemiologic-safety-studies-using-electronic

ClinicalTrials.gov. ClinicalTrials.gov. https://clinicaltrials.gov/

Commission Implementing Regulation (EU) No 520/2012. https://eur-lex.europa.eu/eli/reg_impl/2012/520/oj

Volume 10 of the Rules Governing Medicinal Products in the European Union. Public Health - European Commission. Text. https://ec.europa.eu/health/documents/eudralex/vol-10_en

Developing a Protocol for Observational Comparative Effectiveness Research: A User's Guide | Effective Health Care Program. https://effectivehealthcare.ahrq.gov/products/observational-cer-protocol/research Accessed 27 May 2021

Directive 2001/20/EC. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2001:121:0034:0044:en:PDF

ENCePP Checklist for Study Protocols. ENCePP. http://www.encepp.eu/standards_and_guidances/checkListProtocols.shtml

EU PAS Register. ENCePP. http://www.encepp.eu/encepp/studiesDatabase.jsp

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Guidance for the format and content of the final study report of non-interventional post-authorisation safety studies - EMA. https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guidance-format-content-final-study-report-non-interventional-post-authorisation-safety-studies_en.pdf

Guidance for the format and content of the protocol of non-interventional post-authorisation safety studies - EMA. https://www.ema.europa.eu/en/documents/other/guidance-format-content-protocol-non-interventional-post-authorisation-safety-studies_en.pdf

ISPE Guidelines for Good Pharmacoepidemiology Practices (GPP) - International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Open Science Forum. Open Science Forum. https://osf.io/

Schneeweiss, S., Rassen, J. A., Brown, J. S., Rothman, K. J., Happe, L., Arlett, P., et al. (2019). Graphical Depiction of Longitudinal Study Designs in Health Care Databases. Annals of Internal Medicine, 170(6), 398–406. https://doi.org/10.7326/M18-3079

Chapter 4 – Approaches to data collection

Good pharmacovigilance practices (GPP). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices Accessed 4 May 2021

4.1. Primary data collection

Abenhaim, L., Moride, Y., Brenot, F., Rich, S., Benichou, J., Kurz, X., et al. (1996). Appetite-Suppressant Drugs and the Risk of Primary Pulmonary Hypertension. New England Journal of Medicine, 335(9), 609–616. https://doi.org/10.1056/NEJM199608293350901

Artime, E., Qizilbash, N., Garrido-Estepa, M., Vora, P., Soriano-Gabarró, M., Asiimwe, A., & Pocock, S. (2019). Are risk minimization measures for approved drugs in Europe effective? A systematic review. Expert Opinion on Drug Safety, 18(5), 443–454. https://doi.org/10.1080/14740338.2019.1612875

Artime, E., Qizilbash, N., Herruzo, R., & Garrido-Estepa, M. (2020). Risk Minimisation Evaluation with Process Indicators and Behavioural or Health Outcomes in Europe: Systematic Review. Pharmaceutical Medicine, 34(6), 387–400. https://doi.org/10.1007/s40290-020-00361-w

Check, J., & K. Schutt, R. (2011). Research Methods in Education. Sage Publications.

Clinical trials - Regulation EU No 536/2014. Public Health - European Commission. https://ec.europa.eu/health/human-use/clinical-trials/regulation_en

EudraLex - Volume 10 - Clinical trials guidelines. Public Health - European Commission. https://ec.europa.eu/health/documents/eudralex/vol-10_en

Fayers PM, Machin D. (2007). Quality of Life: the assessment, analysis and interpretation of patient-related outcomes (2nd ed.). Wiley.

Fischer, F., & Kleen, S. (2021). Possibilities, Problems, and Perspectives of Data Collection by Mobile Apps in Longitudinal Epidemiological Studies: Scoping Review. Journal of Medical Internet Research, 23(1), e17691. https://doi.org/10.2196/17691

Good pharmacovigilance practices (GPP). (2018, September 17). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices Accessed 4 May 2021

Groves RM, Fowler FJ, Couper MP et al. (2009). Survey Methodology (2nd ed.). Wiley.

Guideline on good pharmacovigilance practices (GVP) XVI - EMA. https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-good-pharmacovigilance-practices-gvp-module-xvi-risk-minimisation-measures-selection-tools_en.pdf. Accessed 27 May 2021

ICH E6 (R2) Good clinical practice. European Medicines Agency. Text. https://www.ema.europa.eu/en/ich-e6-r2-good-clinical-practice Accessed 27 May 2021

Kaufman, D. W., Rosenberg, L., & Mitchell, A. A. (2001). Signal generation and clarification: use of case–control data. Pharmacoepidemiology and Drug Safety, 10(3), 197–203. https://doi.org/10.1002/pds.571

Kish L. (1995). Survey Sampling. Wiley.

REMS Assessment: Planning and Reporting Guidance for Industry - FDA. https://www.fda.gov/media/119790/download

Research, C. for D. E. and. (2020, May 5). Survey Methodologies to Assess REMS Goals That Relate to Knowledge. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/survey-methodologies-assess-rems-goals-relate-knowledge

Roujeau, J.-C., Kelly, J. P., Naldi, L., Rzany, B., Stern, R. S., Anderson, T., et al. (1995). Medication Use and the Risk of Stevens–Johnson Syndrome or Toxic Epidermal Necrolysis. New England Journal of Medicine, 333(24), 1600–1608. https://doi.org/10.1056/NEJM199512143332404

Shapiro, S. (1983). The design of a study of the drug etiology of agranulocytosis and aplastic anemia. European Journal of Clinical Pharmacology, 24(6), 833–836. https://doi.org/10.1007/BF00607096

Smith, M. Y., Russell, A., Bahri, P., Mol, P. G. M., Frise, S., Freeman, E., & Morrato, E. H. (2018). The RIMES Statement: A Checklist to Assess the Quality of Studies Evaluating Risk Minimization Programs for Medicinal Products. Drug Safety, 41(4), 389–401. https://doi.org/10.1007/s40264-017-0619-x

Standard Definitions Final Dispositions of Case Codes and Outcome Rates for Surveys - AAPOR. https://www.aapor.org/AAPOR_Main/media/publications/Standard-Definitions20169theditionfinal.pdf. Accessed 4 June 2020

4.2. Secondary use of data

Berger, M. L., Sox, H., Willke, R. J., Brixner, D. L., Eichler, H.-G., Goettsch, W., et al. (2017). Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness: Recommendations from the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making. Value in Health, 20(8), 1003–1008. https://doi.org/10.1016/j.jval.2017.08.3019

Cave, A., Kurz, X., & Arlett, P. (2019). Real-World Data for Regulatory Decision Making: Challenges and Possible Solutions for Europe. Clinical Pharmacology & Therapeutics, 106(1), 36–39. https://doi.org/10.1002/cpt.1426

Commissioner, O. of the. (2020, November 24). FDA - Real World Evidence. FDA. https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence Accessed 6 May 2021

ENCePP Resources Database. http://www.encepp.eu/encepp/resourcesDatabase.jsp Accessed 6 May 2021

FDA's Best Practices for Conducting and Reporting Pharmacoepidemiologic Safety Studies Using Electronic Healthcare Data Sets. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/best-practices-conducting-and-reporting-pharmacoepidemiologic-safety-studies-using-electronic

Hall, Gc., Sauer, B., Bourke, A., Brown, Js., Reynolds, Mw., & LoCasale, R. (2012). Guidelines for good database selection and use in pharmacoepidemiology research. Pharmacoepidemiology and drug safety, 21(1). https://doi.org/10.1002/pds.2229

ISPE Guidelines for Good Pharmacoepidemiology Practices (GPP). (n.d.). International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Schneeweiss, S., & Avorn, J. (2005). A review of uses of health care utilization databases for epidemiologic research on therapeutics. Journal of clinical epidemiology, 58(4). https://doi.org/10.1016/j.jclinepi.2004.10.012

Strom B, Kimmel SE, Hennessy S. (2019). Pharmacoepidemiology (6th ed.). Wiley.

4.3. Patient registries

CHMP Guideline on Conduct of Pharmacovigilance for Medicines Used by the Paediatric Population - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/draft-guideline-conduct-pharmacovigilance-medicines-used-paediatric-population_en.pdf

Draft Guideline on registry-based studies - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-registry-based-studies_en.pdf. Accessed 19 May 2021

Emilsson, L., Lindahl, B., Koster, M., Lambe, M., & Ludvigsson, Jf. (2015, January). Review of 103 Swedish Healthcare Quality Registries. Journal of internal medicine. https://doi.org/10.1111/joim.12303

ENCePP Resources Database. http://www.encepp.eu/encepp/resourcesDatabase.jsp

EUnetHTA Joint Action 3. EUnetHTA. https://eunethta.eu/ja3-archive/

EUROCAT. http://www.eurocat-network.eu/

European Platform on Rare Disease Registration. https://eu-rd-platform.jrc.ec.europa.eu

Furu, K., Kieler, H., Haglund, B., Engeland, A., Selmer, R., Stephansson, O., et al. (2015). Selective serotonin reuptake inhibitors and venlafaxine in early pregnancy and risk of birth defects: population based cohort study and sibling design. BMJ, 350. https://doi.org/10.1136/bmj.h1798

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Karanatsios, B., Prang, K.-H., Verbunt, E., Yeung, J. M., Kelaher, M., & Gibbs, P. (2020). Defining key design elements of registry-based randomised controlled trials: a scoping review. Trials, 21(1), 552. https://doi.org/10.1186/s13063-020-04459-z

Lauer, M. S., & D'Agostino, R. B. S. (2013, October 23). The Randomized Registry Trial — The Next Disruptive Technology in Clinical Research? http://dx.doi.org/10.1056/NEJMp1310102. n-perspective. https://doi.org/10.1056/NEJMp1310102

Li, G., Sajobi, T. T., Menon, B. K., Korngut, L., Lowerison, M., James, M., et al. (2016). Registry-based randomized controlled trials- what are the advantages, challenges, and areas for future research? Journal of Clinical Epidemiology, 80, 16–24. https://doi.org/10.1016/j.jclinepi.2016.08.003

McGettigan, P., Olmo, C. A., Plueschke, K., Castillon, M., Zondag, D. N., Bahri, P., et al. (2019). Patient Registries: An Underused Resource for Medicines Evaluation. Drug Safety, 42(11), 1343–1351. https://doi.org/10.1007/s40264-019-00848-9

Methodological guidelines and recommendations for efficient and rational governance of patient registries - PARENT. https://ec.europa.eu/health/sites/default/files/ehealth/docs/patient_registries_guidelines_en.pdf

Norsk pasientregister (NPR). Helsedirektoratet. https://www.helsedirektoratet.no/tema/statistikk-registre-og-rapporter/helsedata-og-helseregistre/norsk-pasientregister-npr

Pacurariu, A., Plueschke, K., Olmo, C. A., & Kurz, X. (2018). Imposed registries within the European postmarketing surveillance system: Extended analysis and lessons learned for regulators. Pharmacoepidemiology and Drug Safety, 27(7), 823–826. https://doi.org/10.1002/pds.4449

Patient registries. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/patient-registries

Pharmachild - PRINTO - Paediatric Rheumatology INternational Trials Organization. https://www.printo.it/projects/ongoing/15

Qualification Opinion - The European Cystic Fibrosis Society Patient Registry (ECFSPR) - EMA. https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-european-cystic-fibrosis-society-patient-registry-ecfspr_en.pdf

Qualification opinion on Cellular therapy module of the European Society for Blood & Marrow Transplantation (EBMT) Registry - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/qualification-opinion-cellular-therapy-module-european-society-blood-marrow-transplantation-ebmt_en.pdf.

Registries for Evaluating Patient Outcomes: A User's Guide: 3rd Edition Addendums (Overview) | Effective Health Care Program. (2018). Agency for Healthcare Research and Quality. https://effectivehealthcare.ahrq.gov/products/registries-guide-3rd-edition-addendum/overview

REQueST Tool and its vision paper – EUnetHTA. EUnetHTA. https://eunethta.eu/request-tool-and-its-vision-paper/

Research, C. for D. E. and. (2020, May 15). Postapproval Pregnancy Safety Studies Guidance for Industry. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/postapproval-pregnancy-safety-studies-guidance-industry

Schmidt, M., Schmidt, S. A. J., Sandegaard, J. L., Ehrenstein, V., Pedersen, L., & Sørensen, H. T. (2015, November 17). The Danish National Patient Registry: a review of content, data quality, and research potential. Clinical Epidemiology. https://doi.org/10.2147/CLEP.S91125

Scientific guidance on post-authorisation efficacy studies - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/scientific-guidance-post-authorisation-efficacy-studies-first-version_en.pdf

Systematic overview of data sources for drug safety in pregnancy research Consultancy EMA/2010/29/CN. http://www.encepp.eu/structure/documents/Data_sources_for_medicines_in_pregnancy_research.pdf

The Swedish National Patient Register. Socialstyrelsen. https://www.socialstyrelsen.se/en/statistics-and-data/registers/register-information/the-national-patient-register/

UK Renal Registry | The Renal Association. https://renal.org/about-us/who-we-are/uk-renal-registry Accessed 19 May 2021

4.4. Spontaneous reports

Al Dweik, R., Stacey, D., Kohen, D., & Yaya, S. (2017). Factors affecting patient reporting of adverse drug reactions: a systematic review. British Journal of Clinical Pharmacology, 83(4), 875–883. https://doi.org/10.1111/bcp.13159

Alvarez, Y., Hidalgo, A., Maignen, F., & Slattery, J. (2010). Validation of Statistical Signal Detection Procedures in EudraVigilance Post-Authorization Data. Drug Safety, 33(6), 475–487. https://doi.org/10.2165/11534410-000000000-00000

EudraVigilance. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/research-development/pharmacovigilance/eudravigilance

Aronson, J. K., & Hauben, M. (2006). Anecdotes that provide definitive evidence. BMJ, 333(7581), 1267–1269. https://doi.org/10.1136/bmj.39036.666389.94

Banovac, M., Candore, G., Slattery, J., Houÿez, F., Haerry, D., Genov, G., & Arlett, P. (2017). Patient Reporting in the EU: Analysis of EudraVigilance Data. Drug Safety, 40(7), 629–645. https://doi.org/10.1007/s40264-017-0534-1

Bate, A., Lindquist, M., & Edwards, I. r. (2008). The application of knowledge discovery in databases to post-marketing drug safety: example of the WHO database. Fundamental & Clinical Pharmacology, 22(2), 127–140. https://doi.org/10.1111/j.1472-8206.2007.00552.x

Characterization of databases (DB) used for signal detection (SD) - IMI PROTECT. http://www.imi-protect.eu/documents/WisniewskietalCharacterisationofdatabasesusedorsignaldetectionposterfinalICPE2012.pdf Accessed 9 June 2021

Detailed guide regarding the EudraVigilance data management activities by the European Medicines Agency - EMA. (n.d.). https://www.ema.europa.eu/en/documents/other/detailed-guide-regarding-eudravigilance-data-management-activities-european-medicines-agency_en.pdf. Accessed 9 June 2021

Kreimeyer, K., Menschik, D., Winiecki, S., Paul, W., Barash, F., Woo, E. J., et al. (2017). Using Probabilistic Record Linkage of Structured and Unstructured Data to Identify Duplicate Cases in Spontaneous Adverse Event Reporting Systems. Drug Safety, 40(7), 571–582. https://doi.org/10.1007/s40264-017-0523-4

Menni, C., Klaser, K., May, A., Polidori, L., Capdevila, J., Louca, P., et al. (2021). Vaccine side-effects and SARS-CoV-2 infection after vaccination in users of the COVID Symptom Study app in the UK: a prospective observational study. The Lancet Infectious Diseases, 0(0). https://doi.org/10.1016/S1473-3099(21)00224-3

Pacurariu, A. C., Coloma, P. M., Haren, A. van, Genov, G., Sturkenboom, M. C. J. M., & Straus, S. M. J. M. (2014). A Description of Signals During the First 18 Months of the EMA Pharmacovigilance Risk Assessment Committee. Drug Safety, 37(12), 1059–1066. https://doi.org/10.1007/s40264-014-0240-1

Postigo, R., Brosch, S., Slattery, J., Haren, A. van, Dogné, J.-M., Kurz, X., et al. (2018). EudraVigilance Medicines Safety Database: Publicly Accessible Data for Research and Public Health Protection. Drug Safety, 41(7), 665–675. https://doi.org/10.1007/s40264-018-0647-1

Practical Aspects of Signal Detection in Pharmacovigilance: Report of CIOMS Working Group VIII • COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. (n.d.). COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. https://cioms.ch/publications/product/practical-aspects-of-signal-detection-in-pharmacovigilance-report-of-cioms-working-group-viii/

Research, C. for D. E. and. (2019, May 22). Questions and Answers on FDA's Adverse Event Reporting System (FAERS). FDA. https://www.fda.gov/drugs/surveillance/questions-and-answers-fdas-adverse-event-reporting-system-faers

The value of patient reporting to the pharmacovigilance system: a systematic review - Inácio - 2017 - British Journal of Clinical Pharmacology - Wiley Online Library. https://bpspubs.onlinelibrary.wiley.com/doi/full/10.1111/bcp.13098 Accessed 9 June 2021

Tregunno, Pm., Fink Db., Fernandez, C., Lazaro-Bengoa, E., & Noren Gn. (2014, April). Performance of probabilistic method to detect duplicate individual case safety reports. Drug safety. https://doi.org/10.1007/s40264-014-0146-y

UMC | VigiBase. https://www.who-umc.org/vigibase/vigibase/

4.5 Social media

Audeh, B., Bellet, F., Beyens, M.-N., Louët, A. L.-L., & Bousquet, C. (2020). Use of Social Media for Pharmacovigilance Activities: Key Findings and Recommendations from the Vigi4Med Project. *Drug Safety*, *43*(9), 835–851. https://doi.org/10.1007/s40264-020-00951-2

Bahri, P. (2020). *Communicating about Risks and Safe Use of Medicines*. Adis Singapore.

Banerjee, A. K., & Ingate, S. (2012). Web-Based Patient-Reported Outcomes in Drug Safety and Risk Management. *Drug Safety*, *35*(6), 437–446. https://doi.org/10.2165/11632390-000000000-00000

Bhattacharya, M., Snyder, S., Malin, M., Truffa, M. M., Marinic, S., Engelmann, R., & Raheja, R. R. (2017). Using Social Media Data in Routine Pharmacovigilance: A Pilot Study to Identify Safety Signals and Patient Perspectives. *Pharmaceutical Medicine*, *31*(3), 167–174. https://doi.org/10.1007/s40290-017-0186-6

Brosch, S., Ferran, A.-M. de, Newbould, V., Farkas, D., Lengsavath, M., & Tregunno, P. (2019). Establishing a Framework for the Use of Social Media in Pharmacovigilance in Europe. *Drug Safety*, *42*(8), 921–930. https://doi.org/10.1007/s40264-019-00811-8

Caster, O., Dietrich, J., Kürzinger, M.-L., Lerch, M., Maskell, S., Norén, G. N., et al. (2018). Assessment of the Utility of Social Media for Broad-Ranging Statistical Signal Detection in Pharmacovigilance: Results from the WEB-RADR Project. *Drug Safety*, *41*(12), 1355–1369. https://doi.org/10.1007/s40264-018-0699-2

Cocos, A., Fiks, A. G., & Masino, A. J. (2017). Deep learning for pharmacovigilance: recurrent neural network architectures for labeling adverse drug reactions in Twitter posts. *Journal of the American Medical Informatics Association*, *24*(4), 813–821. https://doi.org/10.1093/jamia/ocw180

Coloma, P. M., Becker, B., Sturkenboom, M. C. J. M., van Mulligen, E. M., & Kors, J. A. (2015). Evaluating Social Media Networks in Medicines Safety Surveillance: Two Case Studies. *Drug Safety*, *38*(10), 921–930. https://doi.org/10.1007/s40264-015-0333-5

ENISA. Plone Site. https://www.enisa.europa.eu

European Commission's Digital Single Market Glossary. (n.d.). *Shaping Europe's digital future - European Commission*. Text. https://ec.europa.eu/digital-single-market/en/glossary

Gattepaille, L. M., Vidlin, S. H., Bergvall, T., Pierce, C. E., & Ellenius, J. (2020). Prospective Evaluation of Adverse Event Recognition Systems in Twitter: Results from the Web-RADR Project. *Drug Safety*, *43*(8), 797–808. https://doi.org/10.1007/s40264-020-00942-3

General Data Protection Regulation (GDPR) Compliance Guidelines. *GDPR.eu*. https://gdpr.eu/

Good pharmacovigilance practices. *European Medicines Agency*. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Martin, S., Kilich, E., & Dada, S. (2020). "Vaccines for pregnant women…?! Absurd" – Mapping maternal vaccination discourse and stance on social media over six months. *Vaccine*, *38*(42), 6627–6637. https://doi.org/10.1016/j.vaccine.2020.07.072

Opinion 1/2015 Mobile Health-Reconciling technological innovation with data protection - EDPS. https://edps.europa.eu/sites/edp/files/publication/15-05-21_mhealth_en_0.pdf

Powell, G. E., Seifert, H. A., Reblin, T., Burstein, P. J., Blowers, J., Menius, J. A., et al. (2016). Social Media Listening for Routine Post-Marketing Safety Surveillance. *Drug Safety*, *39*(5), 443–454. https://doi.org/10.1007/s40264-015-0385-6

Sloane, R., Osanlou, O., Lewis, D., Bollegala, D., Maskell, S., & Pirmohamed, M. (2015). Social media and pharmacovigilance: A review of the opportunities and challenges. *British Journal of Clinical Pharmacology*, *80*(4), 910–920. https://doi.org/10.1111/bcp.12717

Smartphone Secure Development Guidelines. *ENISA - European Union Agency for Cybersecurity*. Report/Study. https://www.enisa.europa.eu/publications/smartphone-secure-development-guidelines-2016

Stekelenborg, J. van, Ellenius, J., Maskell, S., Bergvall, T., Caster, O., Dasgupta, N., et al. (2019). Recommendations for the Use of Social Media in Pharmacovigilance: Lessons from IMI WEB-RADR. *Drug Safety*, *42*(12), 1393–1407. https://doi.org/10.1007/s40264-019-00858-7

4.6. Research networks for multi-database studies

ADVANCE. VAC4EU. https://vac4eu.org/

Arrhythmogenic potential of drugs | ARITMO Project | FP7 | CORDIS | European Commission. https://cordis.europa.eu/project/id/241679

Background rates of Adverse Events of Special Interest for monitoring COVID-19 vaccines, an ACCESS study. http://www.encepp.eu/encepp/viewResource.htm?id=40361

Becker, B. F. H., Avillach, P., Romio, S., van Mulligen, E. M., Weibel, D., Sturkenboom, M. C. J. M., et al. (2017). CodeMapper: semiautomatic coding of case definitions. A contribution from the ADVANCE project. Pharmacoepidemiology and Drug Safety, 26(8), 998–1005. https://doi.org/10.1002/pds.4245

Candore, G., Hedenmalm, K., Slattery, J., Cave, A., Kurz, X., & Arlett, P. (2020). Can We Rely on Results From IQVIA Medical Research Data UK Converted to the Observational Medical Outcome Partnership Common Data Model? Clinical Pharmacology and Therapeutics, 107(4), 915–925. https://doi.org/10.1002/cpt.1785

CNODES | Canadian Network for Observational Drug Effects Studies. https://www.cnodes.ca/

Comment devenir pharmacien ? - Sos Nsaids Project. https://www.sos-nsaids-project.org/

ConcePTION. https://www.imi-conception.eu/wp-content/uploads/2020/10/ConcePTION-D7.5-Report-on-existing-common-data-models-and-proposals-for-ConcePTION.pdf Accessed 9 June 2021

ConcePTION. https://www.imi-conception.eu/

CONSIGN-International. http://www.encepp.eu/encepp/viewResource.htm?id=40318

COVID-19 vaccine monitoring. VAC4EU. https://vac4eu.org/covid-19-vaccine-monitoring/

Dedman, D., Cabecinha, M., Williams, R., Evans, S. J. W., Bhaskaran, K., & Douglas, I. J. (2020). Approaches for combining primary care electronic health record data from multiple sources: a systematic review of observational studies. BMJ Open, 10(10). https://doi.org/10.1136/bmjopen-2020-037405

E-CORE - Systemic glucocorticoids in the treatment of COVID-19 and risks of adverse outcomes in COVID-19 patients in the primary and secondary care setting. http://www.encepp.eu/encepp/viewResource.htm?id=39816

EMIF. http://www.emif.eu/

ENCePP Resources Database. ENCePP. http://www.encepp.eu/encepp/resourcesDatabase.jsp

EUROmediCAT. https://www.euromedicat.eu/whatiseuromedicat

European Health Data Evidence Network (EHDEN). ehden.eu. https://www.ehden.eu/

Exploring and understanding adverse drug reactions by integrative mining of clinical records and biomedical knowledge | EU-ADR Project | FP7 | CORDIS | European Commission. https://cordis.europa.eu/project/id/215847

Flynn, R., Hedenmalm, K., Murray-Thomas, T., Pacurariu, A., Arlett, P., Shepherd, H., et al. (2020). Ability of Primary Care Health Databases to Assess Medicinal Products Discussed by the European Union Pharmacovigilance Risk Assessment Committee. Clinical Pharmacology & Therapeutics, 107(4), 957–965. https://doi.org/10.1002/cpt.1775

Gini, R., Schuemie, M., Brown, J., Ryan, P., Vacchi, E., Coppola, M., et al. (2016). Data Extraction And Management In Networks Of Observational Health Care Databases For Scientific Research: A Comparison Among EU-ADR, OMOP, Mini-Sentinel And MATRICE Strategies. eGEMs (Generating Evidence & Methods to improve patient outcomes), 4(1). https://doi.org/10.13063/2327-9214.1189

Gini, R., Sturkenboom, M. C. J., Sultana, J., Cave, A., Landi, A., Pacurariu, A., et al. (2020). Different Strategies to Execute Multi-Database Studies for Medicines Surveillance in Real-World Setting: A Reflection on the European Model. Clinical Pharmacology & Therapeutics, 108(2), 228–235. https://doi.org/10.1002/cpt.1833

Monitoring of COVID-19 medicines. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/overview/public-health-threats/coronavirus-disease-covid-19/treatments-vaccines/monitoring-covid-19-medicines-0 Accessed 9 June 2021

Global Research in Paediatrics | GRIP Project | FP7 | CORDIS | European Commission. https://cordis.europa.eu/project/id/261060

Improving Consistency and Understanding of Discrepancies of Findings from Pharmacoepidemiological Studies: the IMI PROTECT Project: Pharmacoepidemiology and Drug Safety: Vol 25, No S1. (n.d.). Wiley Online Library. https://onlinelibrary.wiley.com/toc/10991557/25/S1

Innovative Medicines Initiative. IMI Innovative Medicines Initiative. http://www.imi.europa.eu/

Izem, R., Huang, T.-Y., Hou, L., Pestine, E., Nguyen, M., & Maro, J. C. (2020). Quantifying how small variations in design elements affect risk in an incident cohort study in claims. Pharmacoepidemiology and Drug Safety, 29(1), 84–93. https://doi.org/10.1002/pds.4892

Klungel, O. H., Kurz, X., de Groot, M. C. H., Schlienger, R. G., Tcherny-Lessenot, S., Grimaldi, L., et al. (2016). Multi-centre, multi-database studies with common protocols: lessons learnt from the IMI PROTECT project. Pharmacoepidemiology and Drug Safety, 25(S1), 156–165. https://doi.org/10.1002/pds.3968

Lane, J. C. E., Weaver, J., Kostka, K., Duarte-Salles, T., Abrahao, M. T. F., Alghoul, H., et al. (2020). Risk of hydroxychloroquine alone and in combination with azithromycin in the treatment of rheumatoid arthritis: a multinational, retrospective study. The Lancet Rheumatology, 2(11), e698–e711. https://doi.org/10.1016/S2665-9913(20)30276-9

OHDSI – Observational Health Data Sciences and Informatics. https://www.ohdsi.org/ Accessed 9 June 2021

OMOP Common Data Model – OHDSI. https://www.ohdsi.org/data-standardization/the-common-data-model/

Overhage, J. M., Ryan, P. B., Reich, C. G., Hartzema, A. G., & Stang, P. E. (2012). Validation of a common data model for active safety surveillance research. Journal of the American Medical Informatics Association: JAMIA, 19(1), 54–60. https://doi.org/10.1136/amiajnl-2011-000376

Petersen, I., McCrea, R. L., Sammon, C. J., Osborn, D. P., Evans, S. J., Cowen, P. J., et al. (2016). Risks and benefits of psychotropic medication in pregnancy: cohort studies based on UK electronic primary care health records. Health Technology Assessment, 20(23), 1–176. https://doi.org/10.3310/hta20230

PROTECT. http://www.imi-protect.eu/about.shtml

Research, C. for D. E. and. (2019, October 18). FDA's Sentinel Initiative. FDA. https://www.fda.gov/safety/fdas-sentinel-initiative

Safety Evaluation of Adverse Reactions in Diabetes | SAFEGUARD Project | FP7 | CORDIS | European Commission. https://cordis.europa.eu/project/id/282521

Safety monitoring of Influenza A/H1N1 pandemic vaccines in EudraVigilance. (2011). Vaccine, 29(26), 4378–4387. https://doi.org/10.1016/j.vaccine.2011.04.005

Schuemie, M. J., Ryan, P. B., Pratt, N., Chen, R., You, S. C., Krumholz, H. M., et al. (2020). Large-scale evidence generation and evaluation across a network of databases (LEGEND): assessing validity using hypertension as a case study. Journal of the American Medical Informatics Association, 27(8), 1268–1277. https://doi.org/10.1093/jamia/ocaa124

Sketris, I. S., Carter, N., Traynor, R. L., Watts, D., Kelly, K., & On behalf of the following contributing members of the CNODES Knowledge Translation Team: Pierre Ernst, J. G., Brenda Hemmelgarn, Colleen Metge, Michael Paterson, Robert Platt W. and Gary Teare. (2020). Building a framework for the evaluation of knowledge translation for the Canadian Network for Observational Drug Effect Studies. Pharmacoepidemiology and Drug Safety, 29(S1), 8–25. https://doi.org/10.1002/pds.4738

Slattery, J., & Kurz, X. (2020). Assessing strength of evidence for regulatory decision making in licensing: What proof do we need for observational studies of effectiveness? Pharmacoepidemiology and Drug Safety, 29(10), 1336–1340. https://doi.org/10.1002/pds.5005

Sultana, J., Giorgianni, F., Rea, F., Lucenteforte, E., Lombardi, N., Mugelli, A., et al. (2019). All-cause mortality and antipsychotic use among elderly persons with high baseline cardiovascular and cerebrovascular risk: a multi-center retrospective cohort study in Italy. Expert Opinion on Drug Metabolism & Toxicology, 15(2), 179–188. https://doi.org/10.1080/17425255.2019.1561860

The U.S. Food and Drug Administration's Mini-Sentinel Program: Pharmacoepidemiology and Drug Safety: Vol 21, No S1. (n.d.). Wiley Online Library. https://onlinelibrary.wiley.com/toc/10991557/21/S1

Trifirò, G., Coloma, P. M., Rijnbeek, P. R., Romio, S., Mosseveld, B., Weibel, D., et al. (2014). Combining multiple healthcare databases for postmarketing drug and vaccine safety surveillance: why and how? Journal of Internal Medicine, 275(6), 551–561. https://doi.org/10.1111/joim.12159

VAC4EU. VAC4EU. https://vac4eu.org/

Xu, Y., Zhou, X., Suehs, B. T., Hartzema, A. G., Kahn, M. G., Moride, Y., et al. (2015). A Comparative Assessment of Observational Medical Outcomes Partnership and Mini-Sentinel Common Data Models and Analytics: Implications for Active Drug Safety Surveillance. Drug Safety, 38(8), 749–765. https://doi.org/10.1007/s40264-015-0297-5

Chapter 5 - Study design

5.1. Overview

Pottegård, A., Kurz, X., Moore, N., Christiansen, C. F., & Klungel, O. (2020). Considerations for pharmacoepidemiological analyses in the SARS-CoV-2 pandemic. Pharmacoepidemiology and Drug Safety, 29(8), 825–831. https://doi.org/10.1002/pds.5029

Renoux, C., Azoulay, L., & Suissa, S. (n.d.). Biases in evaluating the safety and effectiveness of drugs for covid-19: designing real-world evidence studies. American Journal of Epidemiology. https://doi.org/10.1093/aje/kwab028

Song, J. W., & Chung, K. C. (2010). Observational Studies: Cohort and Case-Control Studies. Plastic and Reconstructive Surgery, 126(6), 2234–2242. https://doi.org/10.1097/PRS.0b013e3181f44abc

5.2. Types of study design

Burningham, Z., He, T., Teng, C.-C., Zhou, X., Nebeker, J., & Sauer, B. C. (2017). Evaluation of the Case–Crossover (CCO) Study Design for Adverse Drug Event Detection. Drug Safety, 40(9), 789–798. https://doi.org/10.1007/s40264-017-0540-3

Cadarette, S. M., Maclure, M., Delaney, J. A. C., Whitaker, H. J., Hayes, K. N., Wang, S. V., et al. (2021). Control yourself: ISPE-endorsed guidance in the application of self-controlled study designs in pharmacoepidemiology. Pharmacoepidemiology and Drug Safety, 30(6), 671–684. https://doi.org/10.1002/pds.5227

Coutinho, J. M., Zuurbier, S. M., Aramideh, M., & Stam, J. (2012). The Incidence of Cerebral Venous Thrombosis. Stroke, 43(12), 3375–3377. https://doi.org/10.1161/STROKEAHA.112.671453

Farrington, C. P. (2004). Control without separate controls: evaluation of vaccine safety using case-only methods. Vaccine, 22(15–16), 2064–2070. https://doi.org/10.1016/j.vaccine.2004.01.017

Greenland, S. (1996, May). Confounding and exposure trends in case-crossover and case-time-control designs. Epidemiology (Cambridge, Mass.). https://doi.org/10.1097/00001648-199605000-00003

Hallas, J. (1996, September). Evidence of depression provoked by cardiovascular medication: a prescription sequence symmetry analysis. Epidemiology (Cambridge, Mass.). https://pubmed.ncbi.nlm.nih.gov/8862977/

Hallas, J., Pottegård, A., Wang, S., Schneeweiss, S., & Gagne, J. J. (2016). Persistent User Bias in Case-Crossover Studies in Pharmacoepidemiology. American Journal of Epidemiology, 184(10), 761–769. https://doi.org/10.1093/aje/kww079

Impact of vaccination on household transmission of SARS-COV-2 in England - Public Health England. https://khub.net/documents/135939561/390853656/Impact+of+vaccination+on+household+transmission+of+SARS-COV-2+in+England.pdf/35bf4bb1-6ade-d3eb-a39e-9c9b25a8122a?t=1619551571214 Accessed 17 June 2021

Levin, K. A. (2006). Study Design VI - Ecological Studies. Evidence-Based Dentistry, 7(4), 108–108. https://doi.org/10.1038/sj.ebd.6400454

Maclure, M. (1991). The Case-Crossover Design: A Method for Studying Transient Effects on the Risk of Acute Events. American Journal of Epidemiology, 133(2), 144–153. https://doi.org/10.1093/oxfordjournals.aje.a115853

Morales, D. R., Conover, M. M., You, S. C., Pratt, N., Kostka, K., Duarte-Salles, T., et al. (2021). Renin–angiotensin system blockers and susceptibility to COVID-19: an international, open science, cohort analysis. The Lancet Digital Health, 3(2), e98–e114. https://doi.org/10.1016/S2589-7500(20)30289-2

Morgenstern, H. (1995). Ecologic Studies in Epidemiology: Concepts, Principles, and Methods. Annual Review of Public Health, 16(1), 61–81. https://doi.org/10.1146/annurev.pu.16.050195.000425

Rothman K, Greenland S, Lash T. (2008). Modern Epidemiology (3th ed.). Lippincott Williams & Wilkins.

Schuemie, M. J., Ryan, P. B., Man, K. K. C., Wong, I. C. K., Suchard, M. A., & Hripcsak, G. (2019). A plea to stop using the case-control design in retrospective database studies. Statistics in Medicine, 38(22), 4199–4208. https://doi.org/10.1002/sim.8215

Simpson, C. R., Shi, T., Vasileiou, E., Katikireddi, S. V., Kerr, S., Moore, E., et al. (2021). First-dose ChAdOx1 and BNT162b2 COVID-19 vaccines and thrombocytopenic, thromboembolic and hemorrhagic events in Scotland. Nature Medicine, 1–8. https://doi.org/10.1038/s41591-021-01408-4

Suissa, S. (1995). The case-time-control design. Epidemiology, 6(3), 248–253. https://journals.lww.com/epidem/Abstract/1995/05000/THE_CASE_TIME_CONTROL_DESIGN.10.aspx Accessed 17 June 2021

Wang, S., Linkletter, C., Maclure, M., Dore, D., Mor, V., Buka, S., & Wellenius, G. A. (2011). Future Cases as Present Controls to Adjust for Exposure Trend Bias in Case-only Studies. Epidemiology, 22(4), 568–574. https://doi.org/10.1097/EDE.0b013e31821d09cd

Wang, X., & Cheng, Z. (2020). Cross-Sectional Studies: Strengths, Weaknesses, and Recommendations. CHEST, 158(1), S65–S71. https://doi.org/10.1016/j.chest.2020.03.012

Whitaker, H. J., Ghebremichael-Weldeselassie, Y., Douglas, I. J., Smeeth, L., & Farrington, C. P. (2018). Investigating the assumptions of the self-controlled case series method. Statistics in Medicine, 37(4), 643–658. https://doi.org/10.1002/sim.7536

Whitaker, H. J., Paddy Farrington, C., Spiessens, B., & Musonda, P. (2006). Tutorial in biostatistics: the self-controlled case series method. Statistics in Medicine, 25(10), 1768–1797. https://doi.org/10.1002/sim.2302

5.3. Definition and validation of drug exposure, outcomes and covariates

Banda, J. M., Seneviratne, M., Hernandez-Boussard, T., & Shah, N. H. (2018). Advances in Electronic Phenotyping: From Rule-Based Definitions to Machine Learning Models. Annual Review of Biomedical Data Science, 1(1), 53–68. https://doi.org/10.1146/annurev-biodatasci-080917-013315

Brenner, H., & Gefeller, O. (1993). Use of the Positive Predictive Value to Correct for Disease Misclassification in Epidemiologic Studies. American Journal of Epidemiology, 138(11), 1007–1015. https://doi.org/10.1093/oxfordjournals.aje.a116805

Brighton Collaboration. Brighton Collaboration. https://brightoncollaboration.us/

Brunelli, S. M., Gagne, J. J., Huybrechts, K. F., Wang, S. V., Patrick, A. R., Rothman, K. J., & Seeger, J. D. (2013). Estimation using all available covariate information versus a fixed look-back window for dichotomous covariates. Pharmacoepidemiology and Drug Safety, 22(5), 542–550. https://doi.org/10.1002/pds.3434

Carnahan, R. M., & Moores, K. G. (2012). Mini-Sentinel's systematic reviews of validated methods for identifying health outcomes using administrative and claims data: methods and lessons learned. Pharmacoepidemiology and Drug Safety, 21(S1), 82–89. https://doi.org/10.1002/pds.2321

Cber Sentinel Methods - Quantitative Bias Analysis Methodology Development: Sequential Bias Adjustment for Outcome Misclassification - FDA. https://www.sentinelinitiative.org/sites/default/files/Methods/Sentinel_Methods_Sequential_bias.pdf

ClinicalCodes Repository. https://clinicalcodes.rss.mhs.man.ac.uk/

Davé, S., & Petersen, I. (2009). Creating medical and drug code lists to identify cases in primary care databases. Pharmacoepidemiology and Drug Safety, 18(8), 704–707. https://doi.org/10.1002/pds.1770

Greenland, S. (1996). Basic Methods for Sensitivity Analysis of Biases. International Journal of Epidemiology, 25(6), 1107–1116. https://doi.org/10.1093/ije/25.6.1107-a

Hall, G. C., Lanes, S., Bollaerts, K., Zhou, X., Ferreira, G., & Gini, R. (2020). Outcome misclassification: Impact, usual practice in pharmacoepidemiology database studies and an online aid to correct biased estimates of risk ratio or cumulative incidence. Pharmacoepidemiology and Drug Safety, 29(11), 1450–1455. https://doi.org/10.1002/pds.5109

Jurek, A. M., Greenland, S., Maldonado, G., & Church, T. R. (2005). Proper interpretation of non-differential misclassification effects: expectations vs observations. International Journal of Epidemiology, 34(3), 680–687. https://doi.org/10.1093/ije/dyi060

Khan, N. F., Harrison, S. E., & Rose, P. W. (2010). Validity of diagnostic coding within the General Practice Research Database: a systematic review. British Journal of General Practice, 60(572), e128–e136.

Lash, T. L., Fox, M. P., MacLehose, R. F., Maldonado, G., McCandless, L. C., & Greenland, S. (2014). Good practices for quantitative bias analysis. International Journal of Epidemiology, 43(6), 1969–1985. https://doi.org/10.1093/ije/dyu149

Lee, W.-J., Lee, T. A., Pickard, A. S., Shoaibi, A., & Schumock, G. T. (2015). Using linked electronic data to validate algorithms for health outcomes in administrative databases. Journal of Comparative Effectiveness Research, 4(4), 359–366. https://doi.org/10.2217/cer.15.14

Pottegård, A., Christensen, R. dePont, Houji, A., Christiansen, C. B., Paulsen, M. S., Thomsen, J. L., & Hallas, J. (2014). Primary non-adherence in general practice: a Danish register study. European Journal of Clinical Pharmacology, 70(6), 757–763. https://doi.org/10.1007/s00228-014-1677-y

Pye, S. R., Sheppard, T., Joseph, R. M., Lunt, M., Girard, N., Haas, J. S., et al. (2018). Assumptions made when preparing drug exposure data for analysis have an impact on results: An unreported step in pharmacoepidemiology studies. Pharmacoepidemiology and Drug Safety, 27(7), 781–788. https://doi.org/10.1002/pds.4440

Schneeweiss, S., & Avorn, J. (2005). A review of uses of health care utilization databases for epidemiologic research on therapeutics. Journal of Clinical Epidemiology, 58(4), 323–337. https://doi.org/10.1016/j.jclinepi.2004.10.012

5.4. Specific aspects of study design

Abajo, F. J. de, Rodríguez-Martín, S., Lerma, V., Mejía-Abril, G., Aguilar, M., García-Luque, A., et al. (2020). Use of renin–angiotensin–aldosterone system inhibitors and risk of COVID-19 requiring admission to hospital: a case-population study. The Lancet, 395(10238), 1705–1714. https://doi.org/10.1016/S0140-6736(20)31030-8

Bernal, J. L., Cummins, S., & Gasparrini, A. (2017). Interrupted time series regression for the evaluation of public health interventions: a tutorial. International Journal of Epidemiology, 46(1), 348–355. https://doi.org/10.1093/ije/dyw098

Boston, 677 Huntington Avenue, & Ma 02115 +1495-1000. (2012). Causal Inference Book. https://www.hsph.harvard.edu/miguel-hernan/causal-inference-book/

Collier, S., Harvey, C., Brewster, J., Bakerly, N. D., Elkhenini, H. F., Stanciu, R., et al. (2017). Monitoring safety in a phase III real-world effectiveness trial: use of novel methodology in the Salford Lung Study. Pharmacoepidemiology and Drug Safety, 26(3), 344–352. https://doi.org/10.1002/pds.4118

Collins, R., Bowman, L., Landray, M., & Peto, R. (2020). The Magic of Randomization versus the Myth of Real-World Evidence. New England Journal of Medicine. https://doi.org/10.1056/NEJMsb1901642

Dagan, N., Barda, N., Kepten, E., Miron, O., Perchik, S., Katz, M. A., et al. (2021). BNT162b2 mRNA Covid-19 Vaccine in a Nationwide Mass Vaccination Setting. New England Journal of Medicine, 384(15), 1412–1423. https://doi.org/10.1056/NEJMoa2101765

Desai, J. R., Hyde, C. L., Kabadi, S., St Louis, M., Bonato, V., Katrina Loomis, A., et al. (2017). Utilization of Positive and Negative Controls to Examine Comorbid Associations in Observational Database Studies. Medical Care, 55(3), 244–251. https://doi.org/10.1097/MLR.0000000000000640

Dickerman, B. A., García-Albéniz, X., Logan, R. W., Denaxas, S., & Hernán, M. A. (2020). Emulating a target trial in case-control designs: an application to statins and colorectal cancer. International Journal of Epidemiology, 49(5), 1637–1646. https://doi.org/10.1093/ije/dyaa144

Dormuth, C. R., Patrick, A. R., Shrank, W. H., Wright, J. M., Glynn, R. J., Sutherland, J., & Brookhart, M. A. (2009). Statin Adherence and Risk of Accidents. Circulation, 119(15), 2051–2057. https://doi.org/10.1161/CIRCULATIONAHA.108.824151

Draft Master Protocol Assessment of Risk of Safety Outcomes Following COVID-19 Vaccination - FDA. (n.d.). https://www.bestinitiative.org/wp-content/uploads/2021/04/COVID-19-Vaccine-Safety-Inferential-Draft-Master-Protocol.pdf

Dusetzina, S. B., Brookhart, M. A., & Maciejewski, M. L. (2015). Control Outcomes and Exposures for Improving Internal Validity of Nonrandomized Studies. Health Services Research, 50(5), 1432–1451. https://doi.org/10.1111/1475-6773.12279

Eworuke, E., Welch, E. C., Tobenkin, A., & Maro, J. C. (2019). Use of FDA's Sentinel System to Quantify Seizure Risk Immediately Following New Ranolazine Exposure. Drug Safety, 42(7), 897–906. https://doi.org/10.1007/s40264-019-00798-2

Farrington, C. P., Whitaker, H. J., & Hocine, M. N. (2009). Case series analysis for censored, perturbed, or curtailed post-event exposures. Biostatistics, 10(1), 3–16. https://doi.org/10.1093/biostatistics/kxn013

Ford, I., & Norrie, J. (2016). Pragmatic Trials. https://doi.org/10.1056/NEJMra1510059

Franklin, J. M., Patorno, E., Desai, R. J., Glynn, R. J., Martin, D., Quinto, K., et al. (2021). Emulating Randomized Clinical Trials With Nonrandomized Real-World Evidence Studies. Circulation, 143(10), 1002–1013. https://doi.org/10.1161/CIRCULATIONAHA.120.051718

Fröbert, O., Lagerqvist, B., Olivecrona, G. K., Omerovic, E., Gudnason, T., Maeng, M., et al. (2013, October 23). Thrombus Aspiration during ST-Segment Elevation Myocardial Infarction. http://dx.doi.org/10.1056/NEJMoa1308789. research-article. https://doi.org/10.1056/NEJMoa1308789

García-Albéniz, X., Hsu, J., & Hernán, M. A. (2017). The value of explicitly emulating a target trial when using real world evidence: an application to colorectal cancer screening. European Journal of Epidemiology, 32(6), 495–500. https://doi.org/10.1007/s10654-017-0287-2

GetReal - Glossary of Definitions of Common Terms - IMI. https://www.imi-getreal.eu/Portals/1/Documents/01%20deliverables/D1.3%20-%20Revised%20GetReal%20glossary%20-%20FINAL%20updated%20version_25Oct16_webversion.pdf

Group, T. R. C. (2020). Dexamethasone in Hospitalized Patients with Covid-19. New England Journal of Medicine. https://doi.org/10.1056/NEJMoa2021436

Gulmez, S. E., Larrey, D., Pageaux, G.-P., Lignot, S., Lassalle, R., Jové, J., et al. (2013). Transplantation for Acute Liver Failure in Patients Exposed to NSAIDs or Paracetamol (Acetaminophen). Drug Safety, 36(2), 135–144. https://doi.org/10.1007/s40264-012-0013-7

Hallas, J., Whitaker, H., Delaney, J. A., Cadarette, S. M., Pratt, N., & Maclure, M. (n.d.). The Use of active Comparators in self-controlled Designs. American Journal of Epidemiology. https://doi.org/10.1093/aje/kwab110

Hauben, M., Aronson, J. K., & Ferner, R. E. (2016). Evidence of Misclassification of Drug–Event Associations Classified as Gold Standard 'Negative Controls' by the Observational Medical Outcomes Partnership (OMOP). Drug Safety, 39(5), 421–432. https://doi.org/10.1007/s40264-016-0392-2

Hernán, M. A. (2018a). How to estimate the effect of treatment duration on survival outcomes using observational data. BMJ, 360. https://doi.org/10.1136/bmj.k182

Hernán, M. A. (2018b). The C-Word: Scientific Euphemisms Do Not Improve Causal Inference From Observational Data. American Journal of Public Health, 108(5), 616–619. https://doi.org/10.2105/AJPH.2018.304337

Hernán, M. A., & Robins, J. M. (2016). Using Big Data to Emulate a Target Trial When a Randomized Trial Is Not Available. American Journal of Epidemiology, 183(8), 758–764. https://doi.org/10.1093/aje/kwv254

Hernán, M. A., Sauer, B. C., Hernández-Díaz, S., Platt, R., & Shrier, I. (2016). Specifying a target trial prevents immortal time bias and other self-inflicted injuries in observational analyses. Journal of Clinical Epidemiology, 79, 70–75. https://doi.org/10.1016/j.jclinepi.2016.04.014

Horby, P. W., Mafham, M., Bell, J. L., Linsell, L., Staplin, N., Emberson, J., et al. (2020). Lopinavir–ritonavir in patients admitted to hospital with COVID-19 (RECOVERY): a randomised, controlled, open-label, platform trial. The Lancet, 396(10259), 1345–1352. https://doi.org/10.1016/S0140-6736(20)32013-4

Informatics, O. H. D. S. and. Chapter 18 Method Validity | The Book of OHDSI. https://ohdsi.github.io/TheBookOfOhdsi/ Accessed 17 June 2021

Join the PRINCIPLE Trial — PRINCIPLE Trial. https://www.principletrial.org

Kontopantelis, E., Doran, T., Springate, D. A., Buchan, I., & Reeves, D. (2015). Regression based quasi-experimental approach when randomisation is not an option: interrupted time series analysis. The BMJ, 350. https://doi.org/10.1136/bmj.h2750

Lauer, M. S., & D'Agostino, R. B. (2013). The Randomized Registry Trial — The Next Disruptive Technology in Clinical Research? New England Journal of Medicine, 369(17), 1579–1581. https://doi.org/10.1056/NEJMp1310102

Leather, D. A., Jones, R., Woodcock, A., Vestbo, J., Jacques, L., & Thomas, M. (2020). Real-World Data and Randomised Controlled Trials: The Salford Lung Study. Advances in Therapy, 37(3), 977–997. https://doi.org/10.1007/s12325-019-01192-1

Lesko, S. M., & Mitchell, A. A. (1995, March 22). An assessment of the safety of pediatric ibuprofen. A practitioner-based randomized clinical trial. JAMA. https://pubmed.ncbi.nlm.nih.gov/7884951/

Lopes, R. D., Macedo, A. V. S., de Barros E Silva, P. G. M., Moll-Bernardes, R. J., dos Santos, T. M., Mazza, L., et al. (2021). Effect of Discontinuing vs Continuing Angiotensin-Converting Enzyme Inhibitors and Angiotensin II Receptor Blockers on Days Alive and Out of the Hospital in Patients Admitted With COVID-19: A Randomized Clinical Trial. JAMA, 325(3), 254. https://doi.org/10.1001/jama.2020.25864

Loudon, K., Treweek, S., Sullivan, F., Donnan, P., Thorpe, K. E., & Zwarenstein, M. (2015). The PRECIS-2 tool: designing trials that are fit for purpose. BMJ, 350. https://doi.org/10.1136/bmj.h2147

Lund, J. L., Richardson, D. B., & Stürmer, T. (2015). The Active Comparator, New User Study Design in Pharmacoepidemiology: Historical Foundations and Contemporary Application. Current Epidemiology Reports, 2(4), 221–228. https://doi.org/10.1007/s40471-015-0053-5

Maclure, M., Fireman, B., Nelson, J. C., Hua, W., Shoaibi, A., Paredes, A., & Madigan, D. (2012). When should case-only designs be used for safety monitoring of medical products? Pharmacoepidemiology and Drug Safety, 21(S1), 50–61. https://doi.org/10.1002/pds.2330

Mentz, R. J., Hernandez, A. F., Berdan, L. G., Rorick, T., O'Brien, E. C., Ibarra, J. C., et al. (2016). Good Clinical Practice Guidance and Pragmatic Clinical Trials. Circulation, 133(9), 872–880. https://doi.org/10.1161/CIRCULATIONAHA.115.019902

Miettinen, O. S. (1989). The clinical trial as a paradigm for epidemiologic research. Journal of Clinical Epidemiology, 42(6), 491–496. https://doi.org/10.1016/0895-4356(89)90143-1

Moore, N., Gulmez, S. E., Larrey, D., Pageaux, G.-P., Lignot, S., Lassalle, R., et al. (2013). Choice of the denominator in case population studies: event rates for registration for liver transplantation after exposure to NSAIDs in the SALT study in France. Pharmacoepidemiology and Drug Safety, 22(2), 160–167. https://doi.org/10.1002/pds.3371

Nicholas, J. M., Grieve, A. P., & Gulliford, M. C. (2012). Within-person study designs had lower precision and greater susceptibility to bias because of trends in exposure than cohort and nested case–

control designs. Journal of Clinical Epidemiology, 65(4), 384–393. https://doi.org/10.1016/j.jclinepi.2011.09.004

Peach, E. J., Pearce, F. A., Gibson, J., Cooper, A. J., Chen, L.-C., & Knaggs, R. D. (n.d.). Opioids and the Risk of Fracture: A Self-Controlled Case Series Study in the Clinical Practice Research Datalink. American Journal of Epidemiology. https://doi.org/10.1093/aje/kwab042

Pragmatic randomised trials using routine electronic health records: putting them to the test | The BMJ. https://www.bmj.com/content/344/bmj.e55

Rao, S. V., Hess, C. N., & Barham, B. (2014). A Registry-Based Randomized Trial Comparing Radial and Femoral Approaches in Women Undergoing Percutaneous Coronary Intervention: The SAFE-PCI for Women (Study of Access Site for Enhancement of PCI for Women) Trial. JACC: Cardiovascular Interventions, 7(8), 857–867. https://doi.org/10.1016/j.jcin.2014.04.007

Rassen, J. A., & Schneeweiss, S. (2012). Newly marketed medications present unique challenges for nonrandomized comparative effectiveness analyses. Journal of Comparative Effectiveness Research, 1(2), 109–111. https://doi.org/10.2217/cer.12.12

RECOVERY Trial. https://www.recoverytrial.net/

Schneeweiss, S., Gagne, J. J., Glynn, R. J., Ruhl, M., & Rassen, J. A. (2011). Assessing the Comparative Effectiveness of Newly Marketed Medications: Methodological Challenges and Implications for Drug Development. Clinical Pharmacology & Therapeutics, 90(6), 777–790. https://doi.org/10.1038/clpt.2011.235

Schneider, R., Reinau, D., Stoffel, S., Jick, S. s., Meier, C. r., & Spoendlin, J. (2021). Risk of skin cancer in new users of thiazides and thiazide-like diuretics: a cohort study using an active comparator group. British Journal of Dermatology. https://doi.org/10.1111/bjd.19880

Schuemie, M. J., Hripcsak, G., Ryan, P. B., Madigan, D., & Suchard, M. A. (2016). Robust empirical calibration of p-values using observational data. Statistics in Medicine, 35(22), 3883–3888. https://doi.org/10.1002/sim.6977

Schuemie, M. J., Hripcsak, G., Ryan, P. B., Madigan, D., & Suchard, M. A. (2018). Empirical confidence interval calibration for population-level effect estimation studies in observational healthcare data. Proceedings of the National Academy of Sciences, 115(11), 2571–2577.

Schuemie, M. J., Ryan, P. B., DuMouchel, W., Suchard, M. A., & Madigan, D. (2014). Interpreting observational studies: why empirical calibration is needed to correct p-values. Statistics in Medicine, 33(2), 209–218. https://doi.org/10.1002/sim.5925

Staa, T.-P. van, Goldacre, B., Gulliford, M., Cassell, J., Pirmohamed, M., Taweel, A., et al. (2012). Pragmatic randomised trials using routine electronic health records: putting them to the test. BMJ, 344. https://doi.org/10.1136/bmj.e55

Sterne, J. A., Hernán, M. A., Reeves, B. C., Savović, J., Berkman, N. D., Viswanathan, M., et al. (2016). ROBINS-I: a tool for assessing risk of bias in non-randomised studies of interventions. BMJ, 355. https://doi.org/10.1136/bmj.i4919

Strom, B. L., Eng, S. M., Faich, G., Reynolds, R. F., D'Agostino, R. B., Ruskin, J., & Kane, J. M. (2011). Comparative Mortality Associated With Ziprasidone and Olanzapine in Real-World Use Among 18,154 Patients With Schizophrenia: The Ziprasidone Observational Study of Cardiac Outcomes (ZODIAC). American Journal of Psychiatry, 168(2), 193–201. https://doi.org/10.1176/appi.ajp.2010.08040484

Suchard, M. A., Zorych, I., Simpson, S. E., Schuemie, M. J., Ryan, P. B., & Madigan, D. (2013). Empirical Performance of the Self-Controlled Case Series Design: Lessons for Developing a Risk Identification and Analysis System. Drug Safety, 36(1), 83–93. https://doi.org/10.1007/s40264-013-0100-4

Théophile, H., Laporte, J.-R., Moore, N., Martin, K.-L., & Bégaud, B. (2011). The Case-Population Study Design. Drug Safety, 34(10), 861–868. https://doi.org/10.2165/11592140-000000000-00000

Théophile, H., Moore, N., Robinson, P., Bégaud, B., & Pariente, A. (2016). Vaccine Case–Population: A New Method for Vaccine Safety Surveillance. Drug Safety, 39(12), 1197–1209. https://doi.org/10.1007/s40264-016-0449-2

Thorpe, K. E., Zwarenstein, M., Oxman, A. D., Treweek, S., Furberg, C. D., Altman, D. G., et al. (2009). A pragmatic–explanatory continuum indicator summary (PRECIS): a tool to help trial designers. CMAJ, 180(10), E47–E57. https://doi.org/10.1503/cmaj.090523

van Staa, T.-P., Dyson, L., McCann, G., Padmanabhan, S., Belatri, R., Goldacre, B., et al. (2014). The opportunities and challenges of pragmatic point-of-care randomised trials using routinely collected electronic records: evaluations of two exemplar trials. Health Technology Assessment, 18(43), 1–146. https://doi.org/10.3310/hta18430

Weldeselassie, Y. G., Whitaker, H. J., & Farrington, C. P. (2011). Use of the self-controlled case-series method in vaccine safety studies: review and recommendations for best practice. Epidemiology & Infection, 139(12), 1805–1817. https://doi.org/10.1017/S0950268811001531

Whitaker, H. J., Paddy Farrington, C., Spiessens, B., & Musonda, P. (2006). Tutorial in biostatistics: the self-controlled case series method. Statistics in Medicine, 25(10), 1768–1797. https://doi.org/10.1002/sim.2302

Xu, S., Zhang, L., Nelson, J. C., Zeng, C., Mullooly, J., McClure, D., & Glanz, J. (2011). Identifying optimal risk windows for self-controlled case series studies of vaccine safety. Statistics in Medicine, 30(7), 742–752. https://doi.org/10.1002/sim.4125

Zuidgeest, M. G. P., Goetz, I., Groenwold, R. H. H., Irving, E., Thiel, G. J. M. W. van, & Grobbee, D. E. (2017). Series: Pragmatic trials and real world evidence: Paper 1. Introduction. Journal of Clinical Epidemiology, 88, 7–13. https://doi.org/10.1016/j.jclinepi.2016.12.023

Zwarenstein, M., Treweek, S., Gagnier, J. J., Altman, D. G., Tunis, S., Haynes, B., et al. (2008). Improving the reporting of pragmatic trials: an extension of the CONSORT statement. BMJ, 337. https://doi.org/10.1136/bmj.a2390

Chapter 6 - Methods to address bias and confounding

6.1 Bias

Evaluating Medication Effects Outside of Clinical Trials: New-User Designs | American Journal of Epidemiology | Oxford Academic. https://academic.oup.com/aje/article/158/9/915/102549 Accessed 17 June 2021

Gagne, J. J. (2017). New-user designs with conditional propensity scores: a unified complement to the traditional active comparator new-user approach. Pharmacoepidemiology and Drug Safety, 26(4), 469–471. https://doi.org/10.1002/pds.4189

Gerhard, T. (2008). Bias: Considerations for research practice. American Journal of Health-System Pharmacy, 65(22), 2159–2168. https://doi.org/10.2146/ajhp070369

Griffith, G. J., Morris, T. T., Tudball, M. J., Herbert, A., Mancano, G., Pike, L., et al. (2020). Collider bias undermines our understanding of COVID-19 disease risk and severity. Nature Communications, 11. https://doi.org/10.1038/s41467-020-19478-2

Haut, E. R. (2011). Surveillance Bias in Outcomes Reporting. JAMA, 305(23), 2462. https://doi.org/10.1001/jama.2011.822

Horwitz, R. I., & Feinstein, A. R. (1978). Alternative Analytic Methods for Case-Control Studies of Estrogens and Endometrial Cancer. New England Journal of Medicine, 299(20), 1089–1094. https://doi.org/10.1056/NEJM197811162992001

Kiri, V. A., & MacKenzie, G. (2009). RE: "IMMORTAL TIME BIAS IN PHARMACOEPIDEMIOLOGY." American Journal of Epidemiology, 170(5), 667–668. https://doi.org/10.1093/aje/kwp239

Lévesque, L. E., Hanley, J. A., Kezouh, A., & Suissa, S. (2010). Problem of immortal time bias in cohort studies: example using statins for preventing progression of diabetes. BMJ, 340. https://doi.org/10.1136/bmj.b5087

Lewis, M. A. (1999). The transnational study on oral contraceptives and the health of young women. Methods, results, new analyses and the healthy user effect. Human Reproduction Update, 5(6), 707–720. https://doi.org/10.1093/humupd/5.6.707

Lund, J. L., Richardson, D. B., & Stürmer, T. (2015). The Active Comparator, New User Study Design in Pharmacoepidemiology: Historical Foundations and Contemporary Application. Current Epidemiology Reports, 2(4), 221–228. https://doi.org/10.1007/s40471-015-0053-5

Menni, C., Klaser, K., May, A., Polidori, L., Capdevila, J., Louca, P., et al. (2021). Vaccine side-effects and SARS-CoV-2 infection after vaccination in users of the COVID Symptom Study app in the UK: a prospective observational study. The Lancet Infectious Diseases, 0(0). https://doi.org/10.1016/S1473-3099(21)00224-3

Renoux, C., Azoulay, L., & Suissa, S. (n.d.). Biases in evaluating the safety and effectiveness of drugs for covid-19: designing real-world evidence studies. American Journal of Epidemiology. https://doi.org/10.1093/aje/kwab028

Salas, M., Hotman, A., & Stricker, B. H. (1999). Confounding by Indication: An Example of Variation in the Use of Epidemiologic Terminology. American Journal of Epidemiology, 149(11), 981–983. https://doi.org/10.1093/oxfordjournals.aje.a009758

Sharma, M., Nazareth, I., & Petersen, I. (2018). Observational studies of treatment effectiveness: worthwhile or worthless? Clinical Epidemiology. https://doi.org/10.2147/CLEP.S178723

Shrank, W. H., Patrick, A. R., & Brookhart, M. A. (2011). Healthy User and Related Biases in Observational Studies of Preventive Interventions: A Primer for Physicians. Journal of General Internal Medicine, 26(5), 546–550. https://doi.org/10.1007/s11606-010-1609-1

Suissa, S. (2007). Immortal time bias in observational studies of drug effects. Pharmacoepidemiology and Drug Safety, 16(3), 241–249. https://doi.org/10.1002/pds.1357

Suissa, S. (2008). Immeasurable Time Bias in Observational Studies of Drug Effects on Mortality. American Journal of Epidemiology, 168(3), 329–335. https://doi.org/10.1093/aje/kwn135

Suissa, S. (2008). Immortal Time Bias in Pharmacoepidemiology. American Journal of Epidemiology, 167(4), 492–499. https://doi.org/10.1093/aje/kwm324

Suissa, S., & Azoulay, L. (2012). Metformin and the Risk of Cancer: Time-related biases in observational studies. Diabetes Care, 35(12), 2665–2673. https://doi.org/10.2337/dc12-0788

Suissa, S., Moodie, E. E. M., & Dell'Aniello, S. (2017). Prevalent new-user cohort designs for comparative drug effect studies by time-conditional propensity scores. Pharmacoepidemiology and Drug Safety, 26(4), 459–468. https://doi.org/10.1002/pds.4107

Zhou, Z., Rahme, E., Abrahamowicz, M., & Pilote, L. (2005). Survival Bias Associated with Time-to-Treatment Initiation in Drug Effectiveness Evaluation: A Comparison of Methods. American Journal of Epidemiology, 162(10), 1016–1023. https://doi.org/10.1093/aje/kwi307

6.2. Confounding

Ali, M. S., Groenwold, R. H. H., Belitser, S. V., Pestman, W. R., Hoes, A. W., Roes, K. C. B., et al. (2015). Reporting of covariate selection and balance assessment in propensity score analysis is suboptimal: a systematic review. Journal of Clinical Epidemiology, 68(2), 122–131. https://doi.org/10.1016/j.jclinepi.2014.08.011

Ali, M. S., Groenwold, R. H. H., Pestman, W. R., Belitser, S. V., Hoes, A. W., Boer, A. de, & Klungel, O. H. (2013). Time-dependent propensity score and collider-stratification bias: an example of beta 2 - agonist use and the risk of coronary heart disease. European Journal of Epidemiology, 28(4), 291–299. https://doi.org/10.1007/s10654-013-9766-2

Ali, M. S., Groenwold, R. H. H., Pestman, W. R., Belitser, S. V., Roes, K. C. B., Hoes, A. W., et al. (2014). Propensity score balance measures in pharmacoepidemiology: a simulation study. Pharmacoepidemiology and Drug Safety, 23(8), 802–811. https://doi.org/10.1002/pds.3574

Arbogast, P. G., & Ray, W. A. (2009). Use of disease risk scores in pharmacoepidemiologic studies. Statistical Methods in Medical Research, 18(1), 67–80. https://doi.org/10.1177/0962280208092347

Austin, P. C. (2011a). A Tutorial and Case Study in Propensity Score Analysis: An Application to Estimating the Effect of In-Hospital Smoking Cessation Counseling on Mortality. Multivariate Behavioral Research, 46(1), 119–151. https://doi.org/10.1080/00273171.2011.540480

Austin, P. C. (2011b). An Introduction to Propensity Score Methods for Reducing the Effects of Confounding in Observational Studies. Multivariate Behavioral Research, 46(3), 399–424. https://doi.org/10.1080/00273171.2011.568786

Austin, P. C., & Stuart, E. A. (2015). Moving towards best practice when using inverse probability of treatment weighting (IPTW) using the propensity score to estimate causal treatment effects in observational studies. Statistics in Medicine, 34(28), 3661–3679. https://doi.org/10.1002/sim.6607

Baiocchi, M., Cheng, J., & Small, D. S. (2014). Instrumental variable methods for causal inference. Statistics in Medicine, 33(13), 2297–2340. https://doi.org/10.1002/sim.6128

Belitser, S. V., Martens, E. P., Pestman, W. R., Groenwold, R. H. H., de Boer, A., & Klungel, O. H. (2011). Measuring balance and model selection in propensity score methods. Pharmacoepidemiology and Drug Safety, 20(11), 1115–1129. https://doi.org/10.1002/pds.2188

Brookhart, M. A., Rassen, J. A., & Schneeweiss, S. (2010). Instrumental variable methods in comparative safety and effectiveness research. Pharmacoepidemiology and Drug Safety, 19(6), 537–554. https://doi.org/10.1002/pds.1908

Brookhart, M. A., Schneeweiss, S., Rothman, K. J., Glynn, R. J., Avorn, J., & Stürmer, T. (2006). Variable Selection for Propensity Score Models. American Journal of Epidemiology, 163(12), 1149–1156. https://doi.org/10.1093/aje/kwj149

Brookhart, M. A., Wang, P., Solomon, D. H., & Schneeweiss, S. (2006). Evaluating short-term drug effects using a physician-specific prescribing preference as an instrumental variable. Epidemiology (Cambridge, Mass.), 17(3), 268–275. https://doi.org/10.1097/01.ede.0000193606.58671.c5

Cole, S. R., Hernán, M. A., Robins, J. M., Anastos, K., Chmiel, J., Detels, R., et al. (2003). Effect of Highly Active Antiretroviral Therapy on Time to Acquired Immunodeficiency Syndrome or Death using Marginal Structural Models. American Journal of Epidemiology, 158(7), 687–694. https://doi.org/10.1093/aje/kwg206

Desai, R. J., Rothman, K. J., Bateman, B. T., Hernandez-Diaz, S., & Huybrechts, K. F. (2017). A Propensity-score-based Fine Stratification Approach for Confounding Adjustment When Exposure Is Infrequent. Epidemiology, 28(2), 249–257. https://doi.org/10.1097/EDE.0000000000000595

Fewell, Z., Davey Smith, G., & Sterne, J. A. C. (2007). The Impact of Residual and Unmeasured Confounding in Epidemiologic Studies: A Simulation Study. American Journal of Epidemiology, 166(6), 646–655. https://doi.org/10.1093/aje/kwm165

Franklin, J. M., Rassen, J. A., Ackermann, D., Bartels, D. B., & Schneeweiss, S. (2014). Metrics for covariate balance in cohort studies of causal effects. Statistics in Medicine, 33(10), 1685–1699. https://doi.org/10.1002/sim.6058

Garabedian, L. F., Chu, P., Toh, S., Zaslavsky, A. M., & Soumerai, S. B. (2014). Potential Bias of Instrumental Variable Analyses for Observational Comparative Effectiveness Research. Annals of Internal Medicine, 161(2), 131–138. https://doi.org/10.7326/M13-1887

Glynn, R. J., Gagne, J. J., & Schneeweiss, S. (2012). Role of disease risk scores in comparative effectiveness research with emerging therapies. Pharmacoepidemiology and Drug Safety, 21(S2), 138–147. https://doi.org/10.1002/pds.3231

Greenland, S. (2000). An introduction to instrumental variables for epidemiologists. International Journal of Epidemiology, 29(4), 722–729. https://doi.org/10.1093/ije/29.4.722

Grobbee, D. E., & Hoes, A. W. (1997). Confounding and indication for treatment in evaluation of drug treatment for hypertension. BMJ, 315(7116), 1151–1154. https://doi.org/10.1136/bmj.315.7116.1151

Groenwold, R. H. H., de Vries, F., de Boer, A., Pestman, W. R., Rutten, F. H., Hoes, A. W., & Klungel, O. H. (2011). Balance measures for propensity score methods: a clinical example on beta-agonist use and the risk of myocardial infarction. Pharmacoepidemiology and Drug Safety, 20(11), 1130–1137. https://doi.org/10.1002/pds.2251

Ji, X., Small, D. S., Leonard, C. E., & Hennessy, S. (2017). The Trend-in-trend Research Design for Causal Inference. Epidemiology (Cambridge, Mass.), 28(4), 529–536. https://doi.org/10.1097/EDE.0000000000000579

Joffe, M. M., & Rosenbaum, P. R. (1999). Invited Commentary: Propensity Scores. American Journal of Epidemiology, 150(4), 327–333. https://doi.org/10.1093/oxfordjournals.aje.a010011

Karim, M. E., Petkau, J., Gustafson, P., Platt, R. W., & Tremlett, H. (2016). Comparison of Statistical Approaches Dealing with Time-dependent Confounding in Drug Effectiveness Studies. Statistical methods in medical research. https://doi.org/10.1177/0962280216668554

Klungel, O., Martens, E. P., & Psaty, B. M. (2004). Methods to assess intended effects of drug treatment in observational studies are reviewed. Journal of Clinical Epidemiology, 57(12), 1223–1231. https://doi.org/10.1016/j.jclinepi.2004.03.011

Martens, E. P., Pestman, W. R., de Boer, A., Belitser, S. V., & Klungel, O. H. (2006). Instrumental Variables: Application and Limitations. Epidemiology, 17(3), 260–267. https://doi.org/10.1097/01.ede.0000215160.88317.cb

McMahon, A. D. (2003). Approaches to combat with confounding by indication in observational studies of intended drug effects. Pharmacoepidemiology and Drug Safety, 12(7), 551–558. https://doi.org/10.1002/pds.883

Miettinen, O. S. (1976). STRATIFICATION BY A MULTIVARIATE CONFOUNDER SCORE. American Journal of Epidemiology, 104(6), 609–620. https://doi.org/10.1093/oxfordjournals.aje.a112339

Moodie, E. E. M., & Stephens, D. A. (2010). Using Directed Acyclic Graphs to detect limitations of traditional regression in longitudinal studies. International Journal of Public Health, 55(6), 701–703. https://doi.org/10.1007/s00038-010-0184-x

Rassen, J. A., Glynn, R. J., Brookhart, M. A., & Schneeweiss, S. (2011). Covariate Selection in High-Dimensional Propensity Score Analyses of Treatment Effects in Small Samples. American Journal of Epidemiology, 173(12), 1404–1413. https://doi.org/10.1093/aje/kwr001

Rassen, J. A., & Schneeweiss, S. (2012). Using high-dimensional propensity scores to automate confounding control in a distributed medical product safety surveillance system. Pharmacoepidemiology and Drug Safety, 21(S1), 41–49. https://doi.org/10.1002/pds.2328

Rassen, J. A., Shelat, A. A., Franklin, J. M., Glynn, R. J., Solomon, D. H., & Schneeweiss, S. (2013). Matching by Propensity Score in Cohort Studies with Three Treatment Groups. Epidemiology, 24(3), 401–409. https://doi.org/10.1097/EDE.0b013e318289dedf

Rassen, J. A., Shelat, A. A., Myers, J., Glynn, R. J., Rothman, K. J., & Schneeweiss, S. (2012). One-to-many propensity score matching in cohort studies. Pharmacoepidemiology and Drug Safety, 21(S2), 69–80. https://doi.org/10.1002/pds.3263

Robins, J. M., Hernán, M. Á., & Brumback, B. (2000). Marginal Structural Models and Causal Inference in Epidemiology. Epidemiology, 11(5), 550–560. https://journals.lww.com/epidem/Fulltext/2000/09000/Marginal_Structural_Models_and_Causal_Inference_in.11.aspx. Accessed 17 June 2021

Salas, M., Hotman, A., & Stricker, B. H. (1999). Confounding by Indication: An Example of Variation in the Use of Epidemiologic Terminology. American Journal of Epidemiology, 149(11), 981–983. https://doi.org/10.1093/oxfordjournals.aje.a009758

Schmidt, A. F., Klungel, O. H., Groenwold, R. H. H., & Consortium, on behalf of the G. (2016). Adjusting for Confounding in Early Postlaunch Settings: Going Beyond Logistic Regression Models. Epidemiology, 27(1), 133–142. https://doi.org/10.1097/EDE.0000000000000388

Schneeweiss, S. (2006). Sensitivity analysis and external adjustment for unmeasured confounders in epidemiologic database studies of therapeutics. Pharmacoepidemiology and Drug Safety, 15(5), 291–303. https://doi.org/10.1002/pds.1200

Schneeweiss, S. (2018). Automated data-adaptive analytics for electronic healthcare data to study causal treatment effects. Clinical Epidemiology, 10, 771–788. https://doi.org/10.2147/CLEP.S166545

Schneeweiss, S., Rassen, J. A., Glynn, R. J., Avorn, J., Mogun, H., & Brookhart, M. A. (2009). High-dimensional Propensity Score Adjustment in Studies of Treatment Effects Using Health Care Claims Data. Epidemiology, 20(4), 512–522. https://doi.org/10.1097/EDE.0b013e3181a663cc

Software Tools - Sensitivity. (n.d.). https://www.drugepi.org/dope/software#Sensitivity Accessed 17 June 2021

Stürmer, T., Rothman, K. J., Avorn, J., & Glynn, R. J. (2010). Treatment Effects in the Presence of Unmeasured Confounding: Dealing With Observations in the Tails of the Propensity Score Distribution—A Simulation Study. American Journal of Epidemiology, 172(7), 843–854. https://doi.org/10.1093/aje/kwq198

Stürmer, T., Schneeweiss, S., Rothman, K. J., Avorn, J., & Glynn, R. J. (2007). Performance of Propensity Score Calibration—A Simulation Study. American Journal of Epidemiology, 165(10), 1110–1118. https://doi.org/10.1093/aje/kwm074

Swanson, S. A., Robins, J. M., Miller, M., & Hernán, M. A. (2015). Selecting on Treatment: A Pervasive Form of Bias in Instrumental Variable Analyses. American Journal of Epidemiology, 181(3), 191–197. https://doi.org/10.1093/aje/kwu284

Tadrous, M., Gagne, J. J., Stürmer, T., & Cadarette, S. M. (2013). Disease Risk Score (DRS) as a Confounder Summary Method: Systematic Review and Recommendations. Pharmacoepidemiology and drug safety, 22(2), 122–129. https://doi.org/10.1002/pds.3377

Tannen, R. L., Weiner, M. G., & Xie, D. (2008). Replicated studies of two randomized trials of angiotensin- converting enzyme inhibitors: further empiric validation of the 'prior event rate ratio' to adjust for unmeasured confounding by indication. Pharmacoepidemiology and Drug Safety, 17(7), 671–685. https://doi.org/10.1002/pds.1584

Toh, S., García Rodríguez, L. A., & Hernán, M. A. (2011). Confounding adjustment via a semi-automated high-dimensional propensity score algorithm: an application to electronic medical records. Pharmacoepidemiology and Drug Safety, 20(8), 849–857. https://doi.org/10.1002/pds.2152

Uddin, M. J., Groenwold, R. H. H., de Boer, A., Afonso, A. S. M., Primatesta, P., Becker, C., et al. (2016). Evaluating different physician's prescribing preference based instrumental variables in two primary care databases: a study of inhaled long-acting beta2-agonist use and the risk of myocardial infarction. Pharmacoepidemiology and Drug Safety, 25(S1), 132–141. https://doi.org/10.1002/pds.3860

Uddin, M. J., Groenwold, R. H. H., de Boer, A., Belitser, S. V., Roes, K. C. B., Hoes, A. W., & Klungel, O. H. (2014). Performance of instrumental variable methods in cohort and nested case–control studies: a simulation study. Pharmacoepidemiology and Drug Safety, 23(2), 165–177. https://doi.org/10.1002/pds.3555

Uddin, M. J., Groenwold, R. H. H., van Staa, T. P., de Boer, A., Belitser, S. V., Hoes, A. W., et al. (2015). Performance of prior event rate ratio adjustment method in pharmacoepidemiology: a simulation study. Pharmacoepidemiology and Drug Safety, 24(5), 468–477. https://doi.org/10.1002/pds.3724

Williamson, E., Morley, R., Lucas, A., & Carpenter, J. (2012). Propensity scores: From naïve enthusiasm to intuitive understanding. Statistical Methods in Medical Research, 21(3), 273–293. https://doi.org/10.1177/0962280210394483

Witteman, J. C. M., D'Agostino, R. B., Stijnen, T., Kannel, W. B., Cobb, J. C., Ridder, D., et al. (1998). G-estimation of Causal Effects: Isolated Systolic Hypertension and Cardiovascular Death in the Framingham Heart Study. American Journal of Epidemiology, 148(4), 390–401. https://doi.org/10.1093/oxfordjournals.aje.a009658

Wyss, R., Girman, C. J., LoCasale, R. J., Alan Brookhart, M., & Stürmer, T. (2013). Variable selection for propensity score models when estimating treatment effects on multiple outcomes: a simulation study. Pharmacoepidemiology and Drug Safety, 22(1), 77–85. https://doi.org/10.1002/pds.3356

6.3. Missing data

Carpenter, J. R., & Smuk, M. (2021). Missing data: A statistical framework for practice. Biometrical Journal, 63(5), 915–947. https://doi.org/10.1002/bimj.202000196

Evaluation of two-fold fully conditional specification multiple imputation for longitudinal electronic health record data - Welch - 2014 - Statistics in Medicine - Wiley Online Library. https://onlinelibrary.wiley.com/doi/full/10.1002/sim.6184

Gail, M., & Benichou, J. (2000). Encyclopedia of Epidemiologic Methods. Wiley.

Indicator and Stratification Methods for Missing Explanatory Variables in Multiple Linear Regression - Journal of American Statistical Association. https://www.uvm.edu/~statdhtx/StatPages/Missing_Data/Jones.pdf

Lee, K. J., & Carlin, J. B. (2012). Recovery of information from multiple imputation: a simulation study. Emerging Themes in Epidemiology, 9(1), 3. https://doi.org/10.1186/1742-7622-9-3

Little, R., & Rubin, D. (2002). Statistical analysis with missing data (2nd ed.). Wiley.

Moons, K. G. M., Donders, R. A. R. T., Stijnen, T., & Harrell, F. E. (2006). Using the outcome for imputation of missing predictor values was preferred, 59. https://www.sciencedirect.com/science/article/abs/pii/S0895435606000606 Accessed 17 June 2021

Rothman K, Greenland S, Lash T. (2008). Modern Epidemiology (3th ed.). Lippincott Williams & Wilkins.

Royston, P. (2004). Multiple Imputation of Missing Values. The Stata Journal, 4(3), 227–241. https://doi.org/10.1177/1536867X0400400301

Rubin, D. (2004). Multiple Imputation for Nonresponse in Surveys. Wiley.

Schafer, J. (1997). Analysis of Incomplete Multivariate Data. Chapman & Hall/CRC.

The MI Procedure - SAS Institute. https://documentation.sas.com/api/docsets/statug/15.2/content/mi.pdf?locale=en#nameddest=statug_mi_toc Accessed 17 June 2021

Welch, C. A., Petersen, I., Bartlett, J. W., White, I. R., Marston, L., Morris, R. W., et al. (2014). Evaluation of two-fold fully conditional specification multiple imputation for longitudinal electronic health record data. Statistics in Medicine, 33(21), 3725–3737. https://doi.org/10.1002/sim.6184

6.4. Triangulation

Lawlor, D. A., Tilling, K., & Davey Smith, G. (2016). Triangulation in aetiological epidemiology. International Journal of Epidemiology, 45(6), 1866–1886. https://doi.org/10.1093/ije/dyw314

Sujan, A. C., Rickert, M. E., Öberg, A. S., Quinn, P. D., Hernández-Díaz, S., Almqvist, C., et al. (2017). Associations of Maternal Antidepressant Use During the First Trimester of Pregnancy With Preterm Birth, Small for Gestational Age, Autism Spectrum Disorder, and Attention-Deficit/Hyperactivity Disorder in Offspring. JAMA, 317(15), 1553. https://doi.org/10.1001/jama.2017.3413

Chapter 7 - Effect measure modification and interaction

Altman, D. G., & Bland, J. M. (2003). Interaction revisited: the difference between two estimates. BMJ, 326(7382), 219. https://doi.org/10.1136/bmj.326.7382.219

Chandan, J. S., Zemedikun, D. T., Thayakaran, R., Byne, N., Dhalla, S., Acosta-Mena, D., et al. (2021). Nonsteroidal Antiinflammatory Drugs and Susceptibility to COVID-19. Arthritis & Rheumatology, 73(5), 731–739. https://doi.org/10.1002/art.41593

Cohen, J. B., D'Agostino McGowan, L., Jensen, E. T., Rigdon, J., & South, A. M. (2021). Evaluating sources of bias in observational studies of angiotensin-converting enzyme inhibitor/angiotensin II receptor blocker use during COVID-19: beyond confounding. Journal of Hypertension, 39(4), 795–805. https://doi.org/10.1097/HJH.0000000000002706

Exploring interaction effects in small samples increases rates of false-positive and false-negative findings: results from a systematic review and simulation study. (2014). Journal of Clinical Epidemiology, 67(7), 821–829. https://doi.org/10.1016/j.jclinepi.2014.02.008

Knol, M. J., & VanderWeele, T. J. (2012). Recommendations for presenting analyses of effect modification and interaction. International Journal of Epidemiology, 41(2), 514–520. https://doi.org/10.1093/ije/dyr218

Knol, M. J., VanderWeele, T. J., Groenwold, R. H. H., Klungel, O. H., Rovers, M. M., & Grobbee, D. E. (2011). Estimating measures of interaction on an additive scale for preventive exposures. European Journal of Epidemiology, 26(6), 433–438. https://doi.org/10.1007/s10654-011-9554-9

Langan, S. M., Schmidt, S. A., Wing, K., Ehrenstein, V., Nicholls, S. G., Filion, K. B., et al. (2018). The reporting of studies conducted using observational routinely collected health data statement for pharmacoepidemiology (RECORD-PE). BMJ, 363. https://doi.org/10.1136/bmj.k3532

Moher, D., Hopewell, S., Schulz, K. F., Montori, V., Gøtzsche, P. C., Devereaux, P. J., et al. (2010). CONSORT 2010 Explanation and Elaboration: updated guidelines for reporting parallel group randomised trials. Journal of Clinical Epidemiology, 63(8), e1–e37. https://doi.org/10.1016/j.jclinepi.2010.03.004

Tharmarajah, E., Buazon, A., Patel, V., Hannah, J. R., Adas, M., Allen, V. B., et al. (2021). IL-6 inhibition in the treatment of COVID-19: A meta-analysis and meta-regression. Journal of Infection, 82(5), 178–185. https://doi.org/10.1016/j.jinf.2021.03.008

Vandenbroucke, J. P., von Elm, E., Altman, D. G., Gøtzsche, P. C., Mulrow, C. D., Pocock, S. J., et al. (2007). Strengthening the Reporting of Observational Studies in Epidemiology (STROBE): Explanation and Elaboration. Epidemiology, 18(6), 805–835. https://doi.org/10.1097/EDE.0b013e3181577511

VanderWeele, T. J. (2009). On the Distinction Between Interaction and Effect Modification. Epidemiology, 20(6), 863–871. https://doi.org/10.1097/EDE.0b013e3181ba333c

Yola, M., & Lucien, A. (1994). Evidence of the depletion of susceptibles effect in non-experimental pharmacoepidemiologic research. Journal of Clinical Epidemiology, 47(7), 731–737. https://doi.org/10.1016/0895-4356(94)90170-8

Chapter 8 - Systematic reviews and meta-analysis

Amani, B., Khanijahani, A., Amani, B., & Hashemi, P. (2021). Lopinavir/Ritonavir for COVID-19: a Systematic Review and Meta-Analysis. *Journal of Pharmacy & Pharmaceutical Sciences*, *24*, 246–257. https://doi.org/10.18433/jpps31668

Bavishi, C., Whelton, P. K., Mancia, G., Corrao, G., & Messerli, F. H. (2021). Renin–angiotensin-system inhibitors and all-cause mortality in patients with COVID-19: a systematic review and meta-analysis of observational studies. *Journal of Hypertension*, *39*(4), 784–794. https://doi.org/10.1097/HJH.0000000000002784

Chou, R., & Helfand, M. (2005). Challenges in Systematic Reviews That Assess Treatment Harms. *Annals of Internal Medicine*, *142*(12_Part_2), 1090–1099. https://doi.org/10.7326/0003-4819-142-12_Part_2-200506211-00009

Henry, D., Lim, L. L.-Y., Rodriguez, L. A. G., Gutthann, S. P., Carson, J. L., Griffin, M., et al. (1996). Variability in risk of gastrointestinal complications with individual non-steroidal anti-inflammatory drugs: results of a collaborative meta-analysis. *BMJ*, *312*(7046), 1563–1566. https://doi.org/10.1136/bmj.312.7046.1563

Hernandez-Diaz, S., Bateman, B. T., Straub, L., Zhu, Y., Mogun, H., Fischer, M., & Huybrechts, K. F. (n.d.). Safety of Tenofovir Disoproxil Fumarate (TDF) for Pregnant Women facing the COVID-19 Pandemic. *American Journal of Epidemiology*. https://doi.org/10.1093/aje/kwab109

NMA incorporating RWE – RWE Navigator. https://rwe-navigator.eu/use-real-world-evidence/summarise-and-synthesise-rwe/overview-of-evidence-synthesis-and-nma/nma-incorporating-rwe/ Accessed 11 June 2021

Overview of evidence synthesis and network meta-analysis – RWE Navigator. (n.d.). https://rwe-navigator.eu/use-real-world-evidence/summarise-and-synthesise-rwe/overview-of-evidence-synthesis-and-nma/ Accessed 11 June 2021

Sarri, G., Patorno, E., Yuan, H., Guo, J. (Jeff), Bennett, D., Wen, X., et al. (2020). Framework for the synthesis of non-randomised studies and randomised controlled trials: a guidance on conducting a systematic review and meta-analysis for healthcare decision making. *BMJ Evidence-Based Medicine*. https://doi.org/10.1136/bmjebm-2020-111493

Working Group X – Meta-analysis - COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. *COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES*. https://cioms.ch/working_groups/working-group-x/

Chapter 9 - Signal detection methodology and application

Bate, A., & Evans, S. J. W. (2009). Quantitative signal detection using spontaneous ADR reporting. Pharmacoepidemiology and Drug Safety, 18(6), 427–436. https://doi.org/10.1002/pds.1742

Bayer, S., Clark, C., Dang, O., Aberdeen, J., Brajovic, S., Swank, K., et al. (2021). ADE Eval: An Evaluation of Text Processing Systems for Adverse Event Extraction from Drug Labels for Pharmacovigilance. Drug Safety, 44(1), 83–94. https://doi.org/10.1007/s40264-020-00996-3

Berlin, C., Blanch, C., Lewis, D. J., Maladorno, D. D., Michel, C., Petrin, M., et al. (2012). Are all quantitative postmarketing signal detection methods equal? Performance characteristics of logistic regression and Multi-item Gamma Poisson Shrinker. Pharmacoepidemiology and Drug Safety, 21(6), 622–630. https://doi.org/10.1002/pds.2247

Candore, G., Juhlin, K., Manlik, K., Thakrar, B., Quarcoo, N., Seabroke, S., et al. (2015). Comparison of Statistical Signal Detection Methods Within and Across Spontaneous Reporting Databases. Drug Safety, 38(6), 577–587. https://doi.org/10.1007/s40264-015-0289-5

Caster, O., Aoki, Y., Gattepaille, L. M., & Grundmark, B. (2020). Disproportionality Analysis for Pharmacovigilance Signal Detection in Small Databases or Subsets: Recommendations for Limiting False-Positive Associations. Drug Safety, 43(5), 479–487. https://doi.org/10.1007/s40264-020-00911-w

Caster, O., Juhlin, K., Watson, S., & Norén, G. N. (2014). Improved Statistical Signal Detection in Pharmacovigilance by Combining Multiple Strength-of-Evidence Aspects in vigiRank. Drug Safety, 37(8), 617–628. https://doi.org/10.1007/s40264-014-0204-5

Caster, O., Norén, G. N., Madigan, D., & Bate, A. (2010). Large-scale regression-based pattern discovery: The example of screening the WHO global drug safety database. Statistical Analysis and Data Mining: The ASA Data Science Journal, 3(4), 197–208. https://doi.org/10.1002/sam.10078

Caster, O., Norén, G. N., Madigan, D., & Bate, A. (2013). Logistic Regression in Signal Detection: Another Piece Added to the Puzzle. Clinical Pharmacology & Therapeutics, 94(3), 312–312. https://doi.org/10.1038/clpt.2013.107

DuMouchel, W., Yuen, N., Payvandi, N., Booth, W., Rut, A., & Fram, D. (2013). Automated Method for Detecting Increases in Frequency of Spontaneous Adverse Event Reports over Time. Journal of Biopharmaceutical Statistics, 23(1), 161–177. https://doi.org/10.1080/10543406.2013.736809

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

GVP - Module IX Addendum I – Methodological aspects of signal detection from spontaneous reports of suspected adverse reactions - EMA. (n.d.). https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-good-pharmacovigilance-practices-gvp-module-ix-addendum-i-methodological-aspects-signal_en.pdf

Harpaz, R., DuMouchel, W., LePendu, P., Bauer-Mehren, A., Ryan, P., & Shah, N. H. (2013). Performance of Pharmacovigilance Signal Detection Algorithms for the FDA Adverse Event Reporting System. Clinical pharmacology and therapeutics, 93(6). https://doi.org/10.1038/clpt.2013.24

Harpaz, R., DuMouchel, W., & Schuemie, M. (2017). Toward multimodal signal detection of adverse drug reactions. Journal of Biomedical Informatics, 76, 41–49. https://doi.org/10.1016/j.jbi.2017.10.013

Hauben, M., Madigan, D., Gerrits, C. M., Walsh, L., & Puijenbroek, E. P. V. (2005). The role of data mining in pharmacovigilance. Expert Opinion on Drug Safety, 4(5), 929–948. https://doi.org/10.1517/14740338.4.5.929

Holle, L. V., & Bauchau, V. (2014). Use of Logistic Regression to Combine Two Causality Criteria for Signal Detection in Vaccine Spontaneous Report Data. Drug Safety, 37(12), 1047–1057. https://doi.org/10.1007/s40264-014-0237-9

Juhlin, K., Karimi, G., Andér, M., Camilli, S., Dheda, M., Har, T. S., et al. (2015). Using VigiBase to Identify Substandard Medicines: Detection Capacity and Key Prerequisites. Drug Safety, 38(4), 373–382. https://doi.org/10.1007/s40264-015-0271-2

Juhlin, K., Ye, X., Star, K., & Norén, G. N. (2013). Outlier removal to uncover patterns in adverse drug reaction surveillance – a simple unmasking strategy. Pharmacoepidemiology and Drug Safety, 22(10), 1119–1129. https://doi.org/10.1002/pds.3474

Low, Y. S., Caster, O., Bergvall, T., Fourches, D., Zang, X., Norén, G. N., et al. (2016). Cheminformatics-aided pharmacovigilance: application to Stevens-Johnson Syndrome. Journal of the American Medical Informatics Association, 23(5), 968–978. https://doi.org/10.1093/jamia/ocv127

Maignen, F., Hauben, M., Hung, E., Holle, L. V., & Dogne, J.-M. (2014). A conceptual approach to the masking effect of measures of disproportionality. Pharmacoepidemiology and Drug Safety, 23(2), 208–217. https://doi.org/10.1002/pds.3530

Maignen, F., Hauben, M., Hung, E., Van Holle, L., & Dogne, J.-M. (2014). Assessing the extent and impact of the masking effect of disproportionality analyses on two spontaneous reporting systems databases. Pharmacoepidemiology and Drug Safety, 23(2), 195–207. https://doi.org/10.1002/pds.3529

Norén, G. N., & Edwards, I. R. (2009). Modern methods of pharmacovigilance: detecting adverse effects of drugs. Clinical Medicine, 9(5), 486–489. https://doi.org/10.7861/clinmedicine.9-5-486

OHDSI – Observational Health Data Sciences and Informatics. (n.d.). https://www.ohdsi.org/ Accessed 16 June 2021

OMOP Common Data Model – OHDSI. (n.d.). https://www.ohdsi.org/data-standardization/the-common-data-model/

Pacurariu, A. C., Straus, S. M., Trifirò, G., Schuemie, M. J., Gini, R., Herings, R., et al. (2015). Useful Interplay Between Spontaneous ADR Reports and Electronic Healthcare Records in Signal Detection. Drug Safety, 38(12), 1201–1210. https://doi.org/10.1007/s40264-015-0341-5

Pinheiro, L. C., Candore, G., Zaccaria, C., Slattery, J., & Arlett, P. (2018). An algorithm to detect unexpected increases in frequency of reports of adverse events in EudraVigilance. Pharmacoepidemiology and Drug Safety, 27(1), 38–45. https://doi.org/10.1002/pds.4344

Practical Aspects of Signal Detection in Pharmacovigilance: Report of CIOMS Working Group VIII - COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. (n.d.). COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. https://cioms.ch/publications/product/practical-aspects-of-signal-detection-in-pharmacovigilance-report-of-cioms-working-group-viii/

PROTECT Database of adverse drug reactions (EU SPC ADR database). http://www.imi-protect.eu/methodsRep.shtml

PROTECT Home. http://www.imi-protect.eu/about.shtml

Research, C. for D. E. and. (2019, October 18). FDA's Sentinel Initiative. FDA. https://www.fda.gov/safety/fdas-sentinel-initiative

Research, C. for D. E. and. (2020, April 22). Good Pharmacovigilance Practices and Pharmacoepidemiologic Assessment - FDA. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/good-pharmacovigilance-practices-and-pharmacoepidemiologic-assessment Accessed 16 June 2021

Salvo, F., Leborgne, F., Thiessard, F., Moore, N., Bégaud, B., & Pariente, A. (2013). A Potential Event-Competition Bias in Safety Signal Detection: Results from a Spontaneous Reporting Research Database in France. Drug Safety, 36(7), 565–572. https://doi.org/10.1007/s40264-013-0063-5

Scholl, J. H. G., van Hunsel, F. P. A. M., Hak, E., & van Puijenbroek, E. P. (2018). A prediction model-based algorithm for computer-assisted database screening of adverse drug reactions in the

Netherlands. Pharmacoepidemiology and Drug Safety, 27(2), 199–205. https://doi.org/10.1002/pds.4364

Schotland, P., Racz, R., Jackson, D. B., Soldatos, T. G., Levin, R., Strauss, D. G., & Burkhart, K. (2021). Target Adverse Event Profiles for Predictive Safety in the Postmarket Setting. Clinical Pharmacology & Therapeutics, 109(5), 1232–1243. https://doi.org/10.1002/cpt.2074

Screening for adverse reactions in EudraVigilance - EMA. https://www.ema.europa.eu/en/documents/other/screening-adverse-reactions-eudravigilance_en.pdf

Stephenson, W. P., & Hauben, M. (2007). Data mining for signals in spontaneous reporting databases: proceed with caution. Pharmacoepidemiology and Drug Safety, 16(4), 359–365. https://doi.org/10.1002/pds.1323

Summary of product characteristics. (n.d.). European Medicines Agency. https://www.ema.europa.eu/en/glossary/summary-product-characteristics Accessed 16 June 2021

Tatonetti, N. P., Ye, P. P., Daneshjou, R., & Altman, R. B. (2012). Data-Driven Prediction of Drug Effects and Interactions. Science Translational Medicine, 4(125), 125ra31-125ra31. https://doi.org/10.1126/scitranslmed.3003377

Welcome to MedDRA | MedDRA. https://www.meddra.org/

Wisniewski, A. F. Z., Bate, A., Bousquet, C., Brueckner, A., Candore, G., Juhlin, K., et al. (2016). Good Signal Detection Practices: Evidence from IMI PROTECT. Drug Safety, 39(6), 469–490. https://doi.org/10.1007/s40264-016-0405-1

Chapter 10 – The statistical analysis plan

Ärzteblatt, D. Ä. G., Redaktion Deutsches. (n.d.). Avoiding Bias in Observational Studies. Deutsches Ärzteblatt. https://www.aerzteblatt.de/int/archive/article?id=66288

Hiemstra, B., Keus, F., Wetterslev, J., Gluud, C., & van der Horst, I. C. C. (2019). DEBATE-statistical analysis plans for observational studies. BMC Medical Research Methodology, 19(1), 233. https://doi.org/10.1186/s12874-019-0879-5

ICH E9 statistical principles for clinical trials. European Medicines Agency. https://www.ema.europa.eu/en/ich-e9-statistical-principles-clinical-trials

ICH E9(R1) - Addendum on estimands and sensitivity analysis in clinical trials. https://database.ich.org/sites/default/files/E9-R1_Step4_Guideline_2019_1203.pdf

ICH Official website: ICH - Harmonisation for better health. https://www.ich.org/

Thomas, L., & Peterson, E. D. (2012). The Value of Statistical Analysis Plans in Observational Research: Defining High-Quality Research From the Start. JAMA, 308(8), 773. https://doi.org/10.1001/jama.2012.9502

Chapter 11 – Quality management

Blacketer, C., Defalco, F. J., Ryan, P. B., & Rijnbeek, P. R. (2021). Increasing Trust in Real-World Evidence Through Evaluation of Observational Data Quality. medRxiv, 2021.03.25.21254341. https://doi.org/10.1101/2021.03.25.21254341

Blomgren, K. J., Sundström, A., Steineck, G., & Wiholm, B.-E. (2006). Interviewer Variability – Quality Aspects in a Case–Control Study. European Journal of Epidemiology, 21(4), 267–277. https://doi.org/10.1007/s10654-006-0017-7

Cave, A., Kurz, X., & Arlett, P. (2019). Real-World Data for Regulatory Decision Making: Challenges and Possible Solutions for Europe. Clinical Pharmacology & Therapeutics, 106(1), 36–39. https://doi.org/10.1002/cpt.1426

Characterizing RWD Quality and Relevancy for Regulatory Purposes - Margolis Center for Health Policy. https://healthpolicy.duke.edu/sites/default/files/2020-03/characterizing_rwd.pdf

Commission Implementing Regulation (EU) No 520/2012. https://eur-lex.europa.eu/eli/reg_impl/2012/520/oj

Draft Guideline on registry-based studies - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-registry-based-studies_en.pdf Accessed 20 May 2021

European Forum for Good Clinical Practice (EFCGP). EFCGP. https://efgcp.eu/Publications.asp?Type=EFGCP%20publications&L1=13&L2=1 Accessed 20 May 2021

Furu, K. (2008). Establishment of the nationwide Norwegian Prescription Database (NorPD) – new opportunities for research in pharmacoepidemiology in Norway. Norsk Epidemiologi, 18(2). https://doi.org/10.5324/nje.v18i2.23

Good pharmacovigilance practices (GPP). (2018, September 17). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Guidelines for Good Pharmacoepidemiology Practices (GPP) - International Society for Pharmacoepidemiology. ISPE. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Herrett, E., Thomas, S. L., Schoonen, W. M., Smeeth, L., & Hall, A. J. (2010). Validation and validity of diagnoses in the General Practice Research Database: a systematic review. British Journal of Clinical Pharmacology, 69(1), 4–14. https://doi.org/10.1111/j.1365-2125.2009.03537.x

Hoffmann, W., Latza, U., Baumeister, S. E., Brünger, M., Buttmann-Schweiger, N., Hardt, J., et al. (2019). Guidelines and recommendations for ensuring Good Epidemiological Practice (GEP): a guideline developed by the German Society for Epidemiology. European Journal of Epidemiology, 34(3), 301–317. https://doi.org/10.1007/s10654-019-00500-x

Informatics, O. H. D. S. and. The Book of OHDSI. https://ohdsi.github.io/TheBookOfOhdsi/

ISO Quality management principles. https://www.iso.org/files/live/sites/isoorg/files/store/en/PUB100080.pdf. Accessed 20 May 2021

ISO 9000:2015. ISO. https://www.iso.org/cms/render/live/en/sites/isoorg/contents/data/standard/04/54/45481.html

ICH E6 (R2) Good clinical practice. European Medicines Agency. https://www.ema.europa.eu/en/ich-e6-r2-good-clinical-practice

ICH E8 General considerations for clinical studies. European Medicines Agency. https://www.ema.europa.eu/en/ich-e8-general-considerations-clinical-studies

Kahn, M. G., Callahan, T. J., Barnard, J., Bauck, A. E., Brown, J., Davidson, B. N., et al. (2016). A Harmonized Data Quality Assessment Terminology and Framework for the Secondary Use of Electronic

Health Record Data. eGEMs (Generating Evidence & Methods to improve patient outcomes), 4(1). https://doi.org/10.13063/2327-9214.1244

Kiran, D. R. (2016). Total Quality Management - 1st Edition (1st ed.). BSP Books, Elsevier. https://www.elsevier.com/books/total-quality-management/kiran/978-0-12-811035-5 Accessed 28 May 2020

OHDSI – Observational Health Data Sciences and Informatics. OHDSI. https://www.ohdsi.org/

PCORnet. The National Patient-Centered Clinical Research Network. https://pcornet.org/data/ Accessed 20 May 2021

Quality Control and Quality Assurance SOP - AHSC. https://www.imperial.ac.uk/media/imperial-college/research-and-innovation/research-office/public/RGIT_SOP_025_QAQC_v8.0_19-Oct-2020.pdf

Reflection paper on risk based quality management in clinical trials - EMA. https://www.ema.europa.eu/en/documents/scientific-guideline/reflection-paper-risk-based-quality-management-clinical-trials_en.pdf Accessed 20 May 2021

Registries for Evaluating Patient Outcomes: A User's Guide: 3rd Edition Addendums (Overview) | Effective Health Care Program. (2018). AHRQ. https://effectivehealthcare.ahrq.gov/products/registries-guide-3rd-edition-addendum/overview

Reisch, L. M., Fosse, J. S., Beverly, K., Yu, O., Barlow, W. E., Harris, E. L., et al. (2003). Training, Quality Assurance, and Assessment of Medical Record Abstraction in a Multisite Study. American Journal of Epidemiology, 157(6), 546–551. https://doi.org/10.1093/aje/kwg016

Research, C. for D. E. and. (2020, April 29). Best Practices for Conducting and Reporting Pharmacoepidemiologic Safety Studies Using Electronic Healthcare Data Sets. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/best-practices-conducting-and-reporting-pharmacoepidemiologic-safety-studies-using-electronic

Risk proportionate approaches in clinical trials - European Comission. https://ec.europa.eu/health/sites/default/files/files/eudralex/vol-10/2017_04_25_risk_proportionate_approaches_in_ct.pdf

Sprenger, K., Nickerson, D., Meeker-O'Connell, A., & Morrison, B. W. (2013). Quality by Design in Clinical Trials: A Collaborative Pilot With FDA. Therapeutic Innovation & Regulatory Science, 47(2), 161–166. https://doi.org/10.1177/0092861512458909

Theobald, K., Capan, M., Herbold, M., Schinzel, S., & Hundt, F. (2009). Quality assurance in non-interventional studies. GMS German Medical Science, 7. https://doi.org/10.3205/000088

Whitney, C. W., Lind, B. K., & Wahl, P. W. (1998). Quality Assurance and Quality Control in Longitudinal Studies. Epidemiologic Reviews, 20(1), 71–80. https://doi.org/10.1093/oxfordjournals.epirev.a017973

Chapter 12 – Dissemination and communication of study results

Benchimol, E. I., Moher, D., Ehrenstein, V., & Langan, S. M. (2020). Retraction of COVID-19 Pharmacoepidemiology Research Could Have Been Avoided by Effective Use of Reporting Guidelines. Clinical Epidemiology. https://doi.org/10.2147/CLEP.S288677

ClinicalTrials.gov. https://clinicaltrials.gov/

Cohen, J., Da, K., Dg, A., De, B., Ca, G., L, H., et al. (2016). STARD 2015 guidelines for reporting diagnostic accuracy studies: explanation and elaboration. BMJ open, 6(11). https://doi.org/10.1136/bmjopen-2016-012799

ENCePP Checklist for Study Protocols. ENCePP. http://www.encepp.eu/standards_and_guidances/checkListProtocols.shtml

ENCePP Code of Conduct. ENCePP. http://www.encepp.eu/code_of_conduct/index.shtml

EU PAS Register. http://www.encepp.eu/encepp_studies/indexRegister.shtml

FDA's Best Practices for Conducting and Reporting Pharmacoepidemiologic Safety Studies Using Electronic Healthcare Data Sets. (2020, April 29). U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/best-practices-conducting-and-reporting-pharmacoepidemiologic-safety-studies-using-electronic

Good pharmacovigilance practices. (2018, September 17). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

GRACE Principles - Good ReseArch for Comparative Effectiveness. GRACE. https://www.graceprinciples.org/

Guidance for the format and content of the final study report of non-interventional post-authorisation safety studies - EMA. https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guidance-format-content-final-study-report-non-interventional-post-authorisation-safety-studies_en.pdf

Guidance for the format and content of the protocol of non-interventional post-authorisation safety studies - EMA. https://www.ema.europa.eu/en/documents/other/guidance-format-content-protocol-non-interventional-post-authorisation-safety-studies_en.pdf

ICMJE. International Committee of Medical Journal Editors. http://www.icmje.org/

ICMJE | Recommendations. (n.d.). International Committee of Medical Journal Editors. http://www.icmje.org/recommendations/

IEA Good Epidemiology Practices (GEP). (2007). IEA Publications. https://ieaweb.org/IEAWeb/Content/IEA_Publications.aspx Accessed 14 May 2020

ISPE Guidelines for Good Pharmacoepidemiology Practices (GPP). (n.d.). International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Kelly, W. N., Arellano, F. M., Barnes, J., Bergman, U., Edwards, I. R., Fernandez, A. M., et al. (2007). Guidelines for submitting adverse event reports for publication. Pharmacoepidemiology and Drug Safety, 16(5), 581–587. https://doi.org/10.1002/pds.1399

Langan, S. M., Schmidt, S. A., Wing, K., Ehrenstein, V., Nicholls, S. G., Filion, K. B., et al. (2018). The reporting of studies conducted using observational routinely collected health data statement for pharmacoepidemiology (RECORD-PE). BMJ, 363. https://doi.org/10.1136/bmj.k3532

Moher, D., Liberati, A., Tetzlaff, J., & Altman, D. G. (2009). Preferred reporting items for systematic reviews and meta-analyses: the PRISMA statement. BMJ, 339. https://doi.org/10.1136/bmj.b2535

Nicholls, S. G., Quach, P., Elm, E. von, Guttmann, A., Moher, D., Petersen, I., et al. (2015). The REporting of Studies Conducted Using Observational Routinely-Collected Health Data (RECORD)

Statement: Methods for Arriving at Consensus and Developing Reporting Guidelines. PLOS ONE, 10(5), e0125620. https://doi.org/10.1371/journal.pone.0125620

Open Science Forum (OSF). (n.d.). https://osf.io/

Schneeweiss, S., Rassen, J. A., Brown, J. S., Rothman, K. J., Happe, L., Arlett, P., et al. (2019). Graphical Depiction of Longitudinal Study Designs in Health Care Databases. Annals of Internal Medicine, 170(6), 398–406. https://doi.org/10.7326/M18-3079

Schulz, K. F., Altman, D. G., & Moher, D. (2010). CONSORT 2010 Statement: updated guidelines for reporting parallel group randomised trials. BMJ, 340. https://doi.org/10.1136/bmj.c332

Simera, I., Moher, D., Hoey, J., Schulz, K. F., & Altman, D. G. (2010). A catalogue of reporting guidelines for health research. European Journal of Clinical Investigation, 40(1), 35–53. https://doi.org/10.1111/j.1365-2362.2009.02234.x

STROBE - Strengthening the Reporting of Observational studies in Epidemiology Statement Guidelines for reporting observational studies. STROBE. https://strobe-statement.org/index.php?id=strobe-publications

Stroup, D. F., Berlin, J. A., Morton, S. C., Olkin, I., Williamson, G. D., Rennie, D., et al. (2000). Meta-analysis of Observational Studies in Epidemiology: A Proposal for Reporting. JAMA, 283(15), 2008–2012. https://doi.org/10.1001/jama.283.15.2008

The EQUATOR Network | Enhancing the QUAlity and Transparency Of Health Research. (n.d.). https://www.equator-network.org/

Wang, S. V., Schneeweiss, S., Berger, M. L., Brown, J., de Vries, F., Douglas, I., et al. (2017). Reporting to Improve Reproducibility and Facilitate Validity Assessment for Healthcare Database Studies V1.0. Pharmacoepidemiology and Drug Safety, 26(9), 1018–1032. https://doi.org/10.1002/pds.4295

WMA Declaration of Helsinki – Ethical Principles for Medical Research Involving Human Subjects. (n.d.). World Medical Association. https://www.wma.net/policies-post/wma-declaration-of-helsinki-ethical-principles-for-medical-research-involving-human-subjects/

Zwarenstein, M., Treweek, S., Gagnier, J. J., Altman, D. G., Tunis, S., Haynes, B., et al. (2008). Improving the reporting of pragmatic trials: an extension of the CONSORT statement. BMJ, 337. https://doi.org/10.1136/bmj.a2390

Chapter 13 - Data protection and ethical aspects

13.1. *Personal data protection in the European Union*

Article 29 Working Party | European Data Protection Board. https://edpb.europa.eu/about-edpb/more-about-edpb/article-29-working-party_en

COMMISSION DIRECTIVE 2005/28/EC. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2005:091:0013:0019:en:PDF

Commission Implementing Regulation (EU) No 520/2012. (n.d.). https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2012:159:0005:0025:EN:PDF

Data Privacy, Medical Record Confidentiality, and Research in the Interest of Public Health - International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/privacy/

EudraLex - EU Legislation. *Public Health - European Commission*. https://ec.europa.eu/health/documents/eudralex_en

Directive 2001/20/EC. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2001:121:0034:0044:en:PDF

EDPB | European Data Protection Board. https://edpb.europa.eu/edpb_en

ENCePP Home Page. http://www.encepp.eu/

European Data Protection Board. https://edpb.europa.eu/about-edpb/about-edpb/members_en

GDPR - General Data Protection Regulation (EU) 2016/679. https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32016R0679

Good pharmacovigilance practices. *European Medicines Agency*. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Guidelines 3/2018 on the territorial scope of the GDPR (Article 3). https://edpb.europa.eu/sites/default/files/files/file1/edpb_guidelines_3_2018_territorial_scope_after_public_consultation_en_1.pdf

Guidelines for Good Pharmacoepidemiology Practices (GPP) - International Society for Pharmacoepidemiology. https://www.pharmacoepi.org/resources/policies/guidelines-08027/

Human medicines: regulatory information. *European Medicines Agency*. https://www.ema.europa.eu/en/human-medicines-regulatory-information

Question and Answers on the interplay between the Clinical Trials Regulation and the General Data Protection Regulation. https://ec.europa.eu/health/sites/default/files/files/documents/qa_clinicaltrials_gdpr_en.pdf

Regulation (EU) 2018/1725. https://edps.europa.eu/sites/default/files/publication/regulation_eu_2018_1725_en.pdf

Regulation (EU) No 536/2014. (n.d.). https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32014R0536&rid=1

13.2. Scientific integrity and ethical conduct

Agency for Healthcare Research and Quality (AHRQ). AHRQ. https://www.ahrq.gov/

CIOMS International Ethical Guidelines for Health-related Research Involving Human. https://cioms.ch/wp-content/uploads/2017/01/WEB-CIOMS-EthicalGuidelines.pdf Accessed 4 June

ENCePP Code of Conduct. ENCePP. http://www.encepp.eu/code_of_conduct/index.shtml

EU PAS Register. http://www.encepp.eu/encepp_studies/indexRegister.shtml

Good pharmacovigilance practices (GPP). (2018, September 17). European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

HMA-EMA Joint Big Data Taskforce Phase II report: 'Evolving Data-Driven Regulation. https://www.ema.europa.eu/en/documents/other/hma-ema-joint-big-data-taskforce-phase-ii-report-evolving-data-driven-regulation_en.pdf

ICMJE. International Committee of Medical Journal Editors. http://www.icmje.org/

ICMJE | Recommendations. International Committee of Medical Journal Editors. http://www.icmje.org/recommendations/

IEA Good Epidemiology Practices (GEP). (2007). IEA Publications. https://ieaweb.org/IEAWeb/Content/IEA_Publications.aspx

Kurz, X., Bauchau, V., Mahy, P., Glismann, S., van der Aa, L. M., & Simondon, F. (2017). The ADVANCE Code of Conduct for collaborative vaccine studies. Vaccine, 35(15), 1844–1855. https://doi.org/10.1016/j.vaccine.2017.02.039

Registries for Evaluating Patient Outcomes: A User's Guide: 3rd Edition | Effective Health Care Program. (n.d.). AHQR. https://effectivehealthcare.ahrq.gov/products/registries-guide-3rd-edition/research/

WMA Declaration of Helsinki – Ethical Principles for Medical Research Involving Human Subjects. World Medical Association. https://www.wma.net/policies-post/wma-declaration-of-helsinki-ethical-principles-for-medical-research-involving-human-subjects/

Chapter 14 - Specific topics

14.1. Comparative effectiveness research

Agency for Healthcare Research and Quality (AHRQ). https://www.ahrq.gov/ Accessed 4 June 2020

AHRQ Comparative Effectiveness Reviews. (2005). Agency for Healthcare Research and Quality (US).

Arbogast, P. G., & Seeger, J. D. (2012, May). Summary Variables in Observational Research: Propensity Scores and Disease Risk Scores | Effective Health Care Program. https://effectivehealthcare.ahrq.gov/products/observational-research-scores/research

Avorn, J. (2007). In Defense of Pharmacoepidemiology — Embracing the Yin and Yang of Drug Research. New England Journal of Medicine, 357(22), 2219–2221. https://doi.org/10.1056/NEJMp0706892

Black, N. (1996). Why we need observational studies to evaluate the effectiveness of health care. BMJ, 312(7040), 1215–1218. https://doi.org/10.1136/bmj.312.7040.1215

COMET Initiative. http://www.comet-initiative.org/

Comparative Effectiveness Research (CER). US National Library of Medicine. https://hsric.nlm.nih.gov/hsric_public/topic/cer/

Concato, J., Shah, N., & Horwitz, R. I. (2000). Randomized, Controlled Trials, Observational Studies, and the Hierarchy of Research Designs. New England Journal of Medicine, 342(25), 1887–1892. https://doi.org/10.1056/NEJM200006223422507

D, S., & J, L. (2009). Explanatory and Pragmatic Attitudes in Therapeutical Trials. Journal of clinical epidemiology. https://doi.org/10.1016/j.jclinepi.2009.01.012

Developing a Protocol for Observational Comparative Effectiveness Research: A User's Guide - Effective Health Care Program. (2013). https://effectivehealthcare.ahrq.gov/products/observational-cer-protocol/research

Es, J., Ba, B., Ba, B., Ns, F., T, G., Cj, K., et al. (2013). The Incident User Design in Comparative Effectiveness Research. Pharmacoepidemiology and drug safety. https://doi.org/10.1002/pds.3334

EUnetHTA. EUnetHTA. https://eunethta.eu/

Gorst, S. L., Gargon, E., Clarke, M., Blazeby, J. M., Altman, D. G., & Williamson, P. R. (2016). Choosing Important Health Outcomes for Comparative Effectiveness Research: An Updated Review and User Survey. PLOS ONE, 11(1), e0146444. https://doi.org/10.1371/journal.pone.0146444

GRACE Principles - Good ReseArch for Comparative Effectiveness. (2016). https://www.graceprinciples.org/

Grading of Recommendations Assessment, Development and Evaluation (GRADE). (n.d.). https://www.gradeworkinggroup.org/

Hc, S., & Sn, G. (2012). The Methods of Comparative Effectiveness Research. Annual review of public health. https://doi.org/10.1146/annurev-publhealth-031811-124610

IMI GetReal - Real-Life Data In Drug Development. (n.d.). https://www.imi-getreal.eu/ A

ISPOR - International Society for Pharmacoeconomics and Outcomes Research. https://www.ispor.org/

J, H., & J, K. (2012). What Is a Rapid Review? A Methodological Exploration of Rapid Reviews in Health Technology Assessments. International journal of evidence-based healthcare. https://doi.org/10.1111/j.1744-1609.2012.00290.x

Loudon, K., Treweek, S., Sullivan, F., Donnan, P., Thorpe, K. E., & Zwarenstein, M. (2015). The PRECIS-2 tool: designing trials that are fit for purpose. BMJ, 350. https://doi.org/10.1136/bmj.h2147

Ma, H., & Jm, R. (2016). Using Big Data to Emulate a Target Trial When a Randomized Trial Is Not Available. American journal of epidemiology. https://doi.org/10.1093/aje/kwv254

Methods Guide for Effectiveness and Comparative Effectiveness Reviews. (2008). Rockville (MD): Agency for Healthcare Research and Quality (US). http://www.ncbi.nlm.nih.gov/books/NBK47095/

Oh, K., Ep, M., Bm, P., De, G., Sd, S., Bh, S., et al. (2004). Methods to Assess Intended Effects of Drug Treatment in Observational Studies Are Reviewed. Journal of clinical epidemiology. https://doi.org/10.1016/j.jclinepi.2004.03.011

PCORI - Patient-Centered Outcomes Research Institute. https://www.pcori.org/

Ray, W. A. (2003). Evaluating Medication Effects Outside of Clinical Trials: New-User Designs. American Journal of Epidemiology, 158(9), 915–920. https://doi.org/10.1093/aje/kwg231

Rj, V., Ho, A., Am, L., E, B., C, B., Rr, A., et al. (2012). Enhancing Electronic Health Record Measurement of Depression Severity and Suicide Ideation: A Distributed Ambulatory Research in Therapeutics Network (DARTNet) Study. Journal of the American Board of Family Medicine : JABFM. https://doi.org/10.3122/jabfm.2012.05.110053

RWE Navigator. https://rwe-navigator.eu/

S, C., D, G., J, L., T, G., & S, C. (2010). Assessing Medication Exposures and Outcomes in the Frail Elderly: Assessing Research Challenges in Nursing Home Pharmacotherapy. Medical care. https://doi.org/10.1097/MLR.0b013e3181de9d10

S, S. (2007). Developments in Post-marketing Comparative Effectiveness Research. Clinical pharmacology and therapeutics, 82(2), 143–156. https://doi.org/10.1038/sj.clpt.6100249

S, S., & J, A. (2005). A Review of Uses of Health Care Utilization Databases for Epidemiologic Research on Therapeutics. Journal of clinical epidemiology. https://doi.org/10.1016/j.jclinepi.2004.10.012

S, T., Jj, G., Ja, R., Bh, F., M, K., & Js, B. (2013). Confounding Adjustment in Comparative Effectiveness Research Conducted Within Distributed Research Networks. Medical care. https://doi.org/10.1097/MLR.0b013e31829b1bb1

Sauer, B. C., Brookhart, A., Roy, J., & Vanderweele, T. (2013). A Review of Covariate Selection for Nonexperimental Comparative Effectiveness Research. Pharmacoepidemiology and drug safety, 22(11), 1139–1145. https://doi.org/10.1002/pds.3506

Schneeweiss, S., Rassen, J. A., Glynn, R. J., Avorn, J., Mogun, H., & Brookhart, M. A. (2009). High-dimensional propensity score adjustment in studies of treatment effects using health care claims data. Epidemiology (Cambridge, Mass.), 20(4), 512–522. https://doi.org/10.1097/EDE.0b013e3181a663cc

Selby, J. V., Forsythe, L., & Sox, H. C. (2015). Stakeholder-Driven Comparative Effectiveness Research: An Update From PCORI. JAMA, 314(21), 2235–2236. https://doi.org/10.1001/jama.2015.15139

Staa, T.-P. van, Goldacre, B., Gulliford, M., Cassell, J., Pirmohamed, M., Taweel, A., et al. (2012). Pragmatic randomised trials using routine electronic health records: putting them to the test. BMJ, 344. https://doi.org/10.1136/bmj.e55

Tadrous, M., Gagne, J. J., Stürmer, T., & Cadarette, S. M. (2013). Disease Risk Score (DRS) as a Confounder Summary Method: Systematic Review and Recommendations. Pharmacoepidemiology and drug safety, 22(2), 122–129. https://doi.org/10.1002/pds.3377

The final version of HTA Core Model® and the Methodological Guidelines for Rapid REA of Pharmaceuticals is now available. (2013, March 8). EUnetHTA. https://eunethta.eu/the-final-version-of-hta-core-model-and-the-methodological-guidelines-for-rapid-rea-of-pharmaceuticals-is-now-available/

Thorpe, K. E., Zwarenstein, M., Oxman, A. D., Treweek, S., Furberg, C. D., Altman, D. G., et al. (2009). A pragmatic–explanatory continuum indicator summary (PRECIS): a tool to help trial designers. CMAJ, 180(10), E47–E57. https://doi.org/10.1503/cmaj.090523

Vandenbroucke, J. P. (2004). When are observational studies as credible as randomised trials? The Lancet, 363(9422), 1728–1731. https://doi.org/10.1016/S0140-6736(04)16261-2

Zwarenstein, M., Treweek, S., Gagnier, J. J., Altman, D. G., Tunis, S., Haynes, B., et al. (2008). Improving the reporting of pragmatic trials: an extension of the CONSORT statement. BMJ, 337. https://doi.org/10.1136/bmj.a2390

14.2. Vaccine safety and effectiveness

A prospective study to evaluate the safety, effectiveness and impact of the RTS, S/AS01E vaccine in young children in sub-Saharan Africa. ENCePP. http://www.encepp.eu/encepp/viewResource.htm?id=38655

ACCESS template protocol for safety of COVID-19 vaccines. ENCePP. http://www.encepp.eu/encepp/viewResource.htm?id=39362

ADVANCE Report on appraisal of vaccine safety methods. https://vac4eu.org/wp-content/uploads/2019/03/ADVANCE_D4-2_appraisal-safety-methods_final_PU.pdf

Adverse events associated with pandemic influenza vaccines: Comparison of the results of a follow-up study with those coming from spontaneous reporting. (2011). Vaccine, 29(3), 519–522. https://doi.org/10.1016/j.vaccine.2010.10.067

Andrews, N. (2012). Epidemiological designs for vaccine safety assessment: Methods and pitfalls. Biologicals, 40(5), 389–392. https://doi.org/10.1016/j.biologicals.2011.08.010

Andrews, N., Kent, A., & Amin-Chowdhury, Z. (2019). Effectiveness of the seven-valent and thirteen-valent pneumococcal conjugate vaccines in England: The indirect cohort design, 2006–2018. Vaccine, 37(32), 4491–4498. https://doi.org/10.1016/j.vaccine.2019.06.071

Andrews, N., Stowe, J., & Thomas, S. L. (2019). The risk of non-specific hospitalised infections following MMR vaccination given with and without inactivated vaccines in the second year of life. Comparative self–controlled case-series study in England. Vaccine, 37(36), 5211–5217. https://doi.org/10.1016/j.vaccine.2019.07.059

Andrews, N., Waight, P. A., Borrow, R., Ladhani, S., George, R. C., Slack, M. P. E., & Miller, E. (2011). Using the Indirect Cohort Design to Estimate the Effectiveness of the Seven Valent Pneumococcal Conjugate Vaccine in England and Wales. PLOS ONE, 6(12), e28435. https://doi.org/10.1371/journal.pone.0028435

B. Black, S., Law, B., & L. Dekker, C. (2021). The critical role of background rates of possible adverse events in the assessment of COVID-19 vaccine safety. Vaccine, 39(19), 2712–2718. https://doi.org/10.1016/j.vaccine.2021.03.016

Background Rates of Adverse Events of Special Interest for COVID-19 Vaccine Safety Monitoring - FDA. https://www.bestinitiative.org/wp-content/uploads/2021/02/C19-Vaccine-Safety-AESI-Background-Rate-Protocol-FINAL-2020.pdf

Background rates of Adverse Events of Special Interest for monitoring COVID-19 vaccines. (n.d.). ENCePP. http://www.encepp.eu/encepp/viewResource.htm?id=40361 Accessed 18 June 2021

Baker, M. A., Baer, B., Kulldorff, M., Zichittella, L., Reindel, R., DeLuccia, S., et al. (2019). Kawasaki disease and 13-valent pneumococcal conjugate vaccination among young children: A self-controlled risk interval and cohort study with null results. PLoS Medicine, 16(7). https://doi.org/10.1371/journal.pmed.1002844

Baker, M. A., Lieu, T. A., Li, L., Hua, W., Qiang, Y., Kawai, A. T., et al. (2015). A Vaccine Study Design Selection Framework for the Postlicensure Rapid Immunization Safety Monitoring Program. American Journal of Epidemiology, 181(8), 608–618. https://doi.org/10.1093/aje/kwu322

Bardenheier, B. H., Gravenstein, S., & Blackman, C. (2021). Adverse events following mRNA SARS-CoV-2 vaccination among U.S. nursing home residents. Vaccine, 39(29), 3844–3851. https://doi.org/10.1016/j.vaccine.2021.05.088

Baum, U., Sundman, J., Jääskeläinen, S., Nohynek, H., Puumalainen, T., & Jokinen, J. (2017). Establishing and maintaining the National Vaccination Register in Finland. Eurosurveillance, 22(17), 30520. https://doi.org/10.2807/1560-7917.ES.2017.22.17.30520

Bauwens, J., Bonhoeffer, J., & Chen, R. T. (2016). Harmonising Immunisation Safety Assessment in Pregnancy. Vaccine, 34(49), 5991–6110. https://www.sciencedirect.com/journal/vaccine/vol/34/issue/49

Bauwens, J., Bonhoeffer, J., & Kochhar, S. (2017). Harmonising Immunisation Safety Assessment in Pregnancy - Part II. Vaccine, 35(48), 6469–6582. https://www.sciencedirect.com/journal/vaccine/vol/35/issue/48/part/PA

Beck, C. R., McKenzie, B. C., Hashim, A. B., Harris, R. C., University of Nottingham Influenza and the ImmunoCompromised (UNIIC) Study Group, A., & Nguyen-Van-Tam, J. S. (2012). Influenza

Vaccination for Immunocompromised Patients: Systematic Review and Meta-analysis by Etiology. The Journal of Infectious Diseases, 206(8), 1250–1259. https://doi.org/10.1093/infdis/jis487

Bernal, J. L., Andrews, N., Gower, C., Robertson, C., Stowe, J., Tessier, E., et al. (2021). Effectiveness of the Pfizer-BioNTech and Oxford-AstraZeneca vaccines on covid-19 related symptoms, hospital admissions, and mortality in older adults in England: test negative case-control study. BMJ, 373. https://doi.org/10.1136/bmj.n1088

Bie, S. de, Verhamme, K. M. C., Straus, S. M. J. M., Stricker, B. H. C., & Sturkenboom, M. C. J. M. (2012). Vaccine-Based Subgroup Analysis in VigiBase. Drug Safety, 35(4), 335–346. https://doi.org/10.2165/11598120-000000000-00000

Biggart, R., Finn, A., & Marlow, R. (2018). Lack of impact of rotavirus vaccination on childhood seizure hospitalizations in England – An interrupted time series analysis. Vaccine, 36(31), 4589–4592. https://doi.org/10.1016/j.vaccine.2018.06.029

Bollaerts, K., Verstraeten, T., & Cohet, C. (2019). Observational studies of non-specific effects of Diphtheria-Tetanus-Pertussis vaccines in low-income countries: Assessing the potential impact of study characteristics, bias and confounding through meta-regression. Vaccine, 37(1), 34–40. https://doi.org/10.1016/j.vaccine.2018.11.049

Bonaiuti, R. (n.d.). DRIVE. DRIVE. http://www.drive-eu.org/

Brighton Collaboration. (n.d.). Brighton Collaboration. https://brightoncollaboration.us/

Broome, C. V., Facklam, R. R., & Fraser, D. W. (1980). Pneumococcal Disease after Pneumococcal Vaccination. New England Journal of Medicine, 303(10), 549–552. https://doi.org/10.1056/NEJM198009043031003

Brown, J. S., Kulldorff, M., Chan, K. A., Davis, R. L., Graham, D., Pettus, P. T., et al. (2007). Early detection of adverse drug events within population-based health networks: application of sequential testing methods. Pharmacoepidemiology and Drug Safety, 16(12), 1275–1284. https://doi.org/10.1002/pds.1509

Castilla, J., Beristain, X., & Martínez-Artola, V. (2012). Effectiveness of rotavirus vaccines in preventing cases and hospitalizations due to rotavirus gastroenteritis in Navarre, Spain. Vaccine, 30(3), 539–543. https://doi.org/10.1016/j.vaccine.2011.11.071

Chodick, G., Tene, L., Patalon, T., Gazit, S., Ben Tov, A., Cohen, D., & Muhsen, K. (2021). Assessment of Effectiveness of 1 Dose of BNT162b2 Vaccine for SARS-CoV-2 Infection 13 to 24 Days After Immunization. JAMA Network Open, 4(6), e2115985. https://doi.org/10.1001/jamanetworkopen.2021.15985

Chrapkowska, C., Galanis, I., & Kark, M. (2020). Validation of the new Swedish vaccination register – Accuracy and completeness of register data. Vaccine, 38(25), 4104–4110. https://doi.org/10.1016/j.vaccine.2020.04.020

CIOMS Guide to Vaccine Safety Communication - COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. (n.d.). COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. https://cioms.ch/publications/product/cioms-guide-vaccine-safety-communication/

Consortium, E. ça suffit ring vaccination trial. (2015). The ring vaccination trial: a novel cluster randomised controlled trial design to evaluate vaccine efficacy and effectiveness during outbreaks, with special reference to Ebola. BMJ, 351. https://doi.org/10.1136/bmj.h3740

Core protocol for population-based database cohort-studies - DRIVE. https://www.drive-eu.org/wp-content/uploads/2018/12/DRIVE_D7.2_Core-protocol-for-population-based-database-cohort-studies_V1.1.pdf

Core protocol for test-negative design studies - DRIVE. https://www.drive-eu.org/wp-content/uploads/2020/09/DRIVE_D7.1.2_Core-protocol-for-test-negative-design-studies.pdf

COVID-19 | European Centre for Disease Prevention and Control. ECDC. https://qap.ecdc.europa.eu/public/extensions/COVID-19/COVID-19.html#global-overview-tab

COVID-19 Vaccine Safety Surveillance: Active Monitoring Master Protocol - FDA. https://www.bestinitiative.org/wp-content/uploads/2021/02/C19-Vaccine-Safety-Protocol-2021.pdf

Covid-19 vaccines: safety surveillance manual. WHO. https://www.who.int/publications-detail-redirect/10665338400

Crowcroft, N. S., & Klein, N. P. (2018). A framework for research on vaccine effectiveness. Vaccine, 36(48), 7286–7293. https://doi.org/10.1016/j.vaccine.2018.04.016

Dagan, N., Barda, N., Kepten, E., Miron, O., Perchik, S., Katz, M. A., et al. (2021). BNT162b2 mRNA Covid-19 Vaccine in a Nationwide Mass Vaccination Setting. New England Journal of Medicine. https://doi.org/10.1056/NEJMoa2101765

Dashboard Background rates of Adverse Events of Special Interest for COVID-19 vaccines - VAC4EU. https://vac4eu.org/covid-19-tool/

De Serres, G., Pilishvili, T., & Link-Gelles, R. (2012). Use of surveillance data to estimate the effectiveness of the 7-valent conjugate pneumococcal vaccine in children less than 5 years of age over a 9 year period. Vaccine, 30(27), 4067–4072. https://doi.org/10.1016/j.vaccine.2012.04.017

De Vito, C., Manzoli, L., & Marzuillo, C. (2007). A systematic review evaluating the potential for bias and the methodological quality of meta-analyses in vaccinology. Vaccine, 25(52), 8794–8806. https://doi.org/10.1016/j.vaccine.2007.10.034

Dhanda, S., Osborne, V., Lynn, E., & Shakir, S. (2020). Postmarketing studies: can they provide a safety net for COVID-19 vaccines in the UK? BMJ Evidence-Based Medicine. https://doi.org/10.1136/bmjebm-2020-111507

Dieleman, J., Romio, S., Johansen, K., Weibel, D., Bonhoeffer, J., & Sturkenboom, M. (2011). Guillain-Barré syndrome and adjuvanted pandemic influenza A (H1N1) 2009 vaccine: multinational case-control study in Europe. BMJ, 343. https://doi.org/10.1136/bmj.d3908

Drolet, M., Bénard, É., Pérez, N., Brisson, M., Ali, H., Boily, M.-C., et al. (2019). Population-level impact and herd effects following the introduction of human papillomavirus vaccination programmes: updated systematic review and meta-analysis. The Lancet, 394(10197), 497–509. https://doi.org/10.1016/S0140-6736(19)30298-3

Dudareva-Vizule, S., Koch, J., Heiden, M. an der, Oberle, D., Keller-Stanislawski, B., & Wichmann, O. (2012). Impact of rotavirus vaccination in regions with low and moderate vaccine uptake in Germany. Human Vaccines & Immunotherapeutics, 8(10), 1407–1415. https://doi.org/10.4161/hv.21593

Effectiveness of rotavirus vaccination – Generic study protocol for retrospective cohort studies based on computerised databases - ECDC. https://www.ecdc.europa.eu/sites/portal/files/media/en/publications/Publications/rotavirus-vaccination-cohort-study-april2013.pdf

Escolano, S., Farrington, C. P., Hill, C., & Tubert-Bitter, P. (2011). Intussusception after Rotavirus Vaccination — Spontaneous Reports. New England Journal of Medicine, 365(22), 2139–2139. https://doi.org/10.1056/NEJMc1107771

Escolano, S., Hill, C., & Tubert-Bitter, P. (2013). A New Self-Controlled Case Series Method for Analyzing Spontaneous Reports of Adverse Events After Vaccination. American Journal of Epidemiology, 178(9), 1496–1504. https://doi.org/10.1093/aje/kwt128

European Centre for Disease Prevention and Control. https://www.ecdc.europa.eu/en

Evaluation of COVID-19 vaccine effectiveness. World Health Organization. https://www.who.int/publications-detail-redirect/WHO-2019-nCoV-vaccine_effectiveness-measurement-2021.1

Evans, S. J. W. (2008). Stratification for Spontaneous Report Databases. Drug Safety, 31(11), 1049–1052. https://doi.org/10.2165/00002018-200831110-00009

Exploring the Feasibility of Conducting Vaccine Effectiveness Studies in Sentinel's PRISM Program - FDA. https://www.sentinelinitiative.org/sites/default/files/Methods/Sentinel_PRISM_Vaccine_Effectiveness_White_Paper.pdf

Farrington, C. P. (2004). Control without separate controls: evaluation of vaccine safety using case-only methods. Vaccine, 22(15–16), 2064–2070. https://doi.org/10.1016/j.vaccine.2004.01.017

Farrington, C. P., Firth, M. J., Moulton, L. H., Ravn, H., Andersen, P. K., Evans, S., & on behalf of the Working Group on Non-specific Effects of Vaccines. (2009). Epidemiological studies of the non-specific effects of vaccines: II – methodological issues in the design and analysis of cohort studies. Tropical Medicine & International Health, 14(9), 977–985. https://doi.org/10.1111/j.1365-3156.2009.02302.x

Fell, D. B., Dimitris, M. C., & Hutcheon, J. A. (2021). Guidance for design and analysis of observational studies of fetal and newborn outcomes following COVID-19 vaccination during pregnancy. Vaccine, 39(14), 1882–1886. https://doi.org/10.1016/j.vaccine.2021.02.070

Fine, P. E. M., Williams, T. N., Aaby, P., Källander, K., Moulton, L. H., Flanagan, K. L., et al. (2009). Epidemiological studies of the 'non-specific effects' of vaccines: I – data collection in observational studies. Tropical Medicine & International Health, 14(9), 969–976. https://doi.org/10.1111/j.1365-3156.2009.02301.x

Glanz, J. M., McClure, D. L., Xu, S., Hambidge, S. J., Lee, M., Kolczak, M. S., et al. (2006). Four different study designs to evaluate vaccine safety were equally validated with contrasting limitations. Journal of Clinical Epidemiology, 59(8), 808–818. https://doi.org/10.1016/j.jclinepi.2005.11.012

Greene, S. K., Kulldorff, M., Lewis, E. M., Li, R., Yin, R., Weintraub, E. S., et al. (2010). Near Real-Time Surveillance for Influenza Vaccine Safety: Proof-of-Concept in the Vaccine Safety Datalink Project. American Journal of Epidemiology, 171(2), 177–188. https://doi.org/10.1093/aje/kwp345

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices

Haas, E. J., Angulo, F. J., McLaughlin, J. M., Anis, E., Singer, S. R., Khan, F., et al. (2021). Impact and effectiveness of mRNA BNT162b2 vaccine against SARS-CoV-2 infections and COVID-19 cases, hospitalisations, and deaths following a nationwide vaccination campaign in Israel: an observational study using national surveillance data. The Lancet, 397(10287), 1819–1829. https://doi.org/10.1016/S0140-6736(21)00947-8

Halloran, M. E., Haber, M., Longini, I. M., & Struchiner, C. J. (1991). Direct and Indirect Effects in Vaccine Efficacy and Effectiveness. American Journal of Epidemiology, 133(4), 323–331. https://doi.org/10.1093/oxfordjournals.aje.a115884

Halloran, M. E., Longini, I. M., & Struchiner, C. J. (n.d.). Design and Analysis of Vaccine Studies | M. Elizabeth Halloran | Springer. https://www.springer.com/gp/book/9780387403137 Accessed 22 June 2021

Hanquet, G., Valenciano, M., Simondon, F., & Moren, A. (2013). Vaccine effects and impact of vaccination programmes in post-licensure studies. Vaccine, 31(48), 5634–5642. https://doi.org/10.1016/j.vaccine.2013.07.006

Heffernan, J. M., & Keeling, M. J. (2009). Implications of vaccination and waning immunity. Proceedings of the Royal Society B: Biological Sciences, 276(1664), 2071–2080. https://doi.org/10.1098/rspb.2009.0057

Heininger, U., Holm, K., Caplanusi, I., & Bailey, S. R. (2017). Guide to active vaccine safety surveillance: Report of CIOMS working group on vaccine safety – executive summary. Vaccine, 35(32), 3917–3921. https://doi.org/10.1016/j.vaccine.2017.06.033

Hennessy, S., Strom, B. L., Bilker, W. B., Zhengle, L., Chao-Min, W., Hui-Lian, L., et al. (1996). Effectiveness of live-attenuated Japanese encephalitis vaccine (SA14-14-2): a case-control study. The Lancet, 347(9015), 1583–1586. https://doi.org/10.5555/uri:pii:S0140673696910752

Hocine, M., Farrington, C., Touzé, E., Whitaker, H., Fourrier, A., Moreau, T., & Tubert-Bitter, P. (2007). Hepatitis B vaccination and first central nervous system demyelinating events: reanalysis of a case-control study using the self-controlled case series method. Vaccine. https://doi.org/10.1016/j.vaccine.2007.05.032

Hviid, A., Svanström, H., Scheller, N. M., Grönlund, O., Pasternak, B., & Arnheim-Dahlström, L. (2018). Human papillomavirus vaccination of adult women and risk of autoimmune and neurological diseases. Journal of Internal Medicine, 283(2), 154–165. https://doi.org/10.1111/joim.12694

Impact of rotavirus vaccination - Generic study protocol - ECDC. https://www.ecdc.europa.eu/sites/portal/files/media/en/publications/Publications/Rotavirus-impact-vaccination-April-2013.pdf

Impact of vaccination on household transmission of SARS-COV-2 in England - Public Health England. https://khub.net/documents/135939561/390853656/Impact+of+vaccination+on+household+transmission+of+SARS-COV-2+in+England.pdf/35bf4bb1-6ade-d3eb-a39e-9c9b25a8122a?t=1619551571214

Jackson, M. L., & Nelson, J. C. (2013). The test-negative design for estimating influenza vaccine effectiveness. Vaccine, 31(17), 2165–2168. https://doi.org/10.1016/j.vaccine.2013.02.053

Jefferson, T., Rivetti, D., Rivetti, A., Rudin, M., Pietrantonj, C. D., & Demicheli, V. (2005). Efficacy and effectiveness of influenza vaccines in elderly people: a systematic review. The Lancet, 366(9492), 1165–1174. https://doi.org/10.1016/S0140-6736(05)67339-4

Klein, N. P. (2011). Vaccine safety in special populations. Human Vaccines, 7(2), 269–271. https://doi.org/10.4161/hv.7.2.13860

Kulldorff, M., Dashevsky, I., Avery, T. R., Chan, A. K., Davis, R. L., Graham, D., et al. (2013). Drug safety data mining with a tree-based scan statistic. Pharmacoepidemiology and Drug Safety, 22(5), 517–523. https://doi.org/10.1002/pds.3423

Lasky, T., McMahon, A. W., & Hua, W. (2021). Methodologic approaches in studies using real-world data (RWD) to measure pediatric safety and effectiveness of vaccines administered to pregnant women: A scoping review. Vaccine, 39(29), 3814–3824. https://doi.org/10.1016/j.vaccine.2021.05.071

Layton, D., & Shakir, S. A. W. (2015). Specialist Cohort Event Monitoring Studies: A New Study Method for Risk Management in Pharmacovigilance. Drug Safety, 38(2), 153–163. https://doi.org/10.1007/s40264-014-0260-x

Leino, T., Ollgren, J., & Salo, H. (2012). First year experience of rotavirus immunisation programme in Finland. Vaccine, 31(1), 176–182. https://doi.org/10.1016/j.vaccine.2012.10.068

Leite, A., Andrews, N. J., & Thomas, S. L. (2016). Near real-time vaccine safety surveillance using electronic health records—a systematic review of the application of statistical methods. Pharmacoepidemiology and Drug Safety, 25(3), 225–237. https://doi.org/10.1002/pds.3966

Li, X., Ostropolets, A., Makadia, R., Shoaibi, A., Rao, G., Sena, A. G., et al. (2021). Characterising the background incidence rates of adverse events of special interest for covid-19 vaccines in eight countries: multinational network cohort study. BMJ, 373. https://doi.org/10.1136/bmj.n1435

Lusignan, S. de, Santos, G. D., Correa, A., Haguinet, F., Yonova, I., Lair, F., et al. (2017). Post-authorisation passive enhanced safety surveillance of seasonal influenza vaccines: protocol of a pilot study in England. BMJ Open, 7(5), e015469. https://doi.org/10.1136/bmjopen-2016-015469

Mahaux, O., Bauchau, V., & Van Holle, L. (2016). Pharmacoepidemiological considerations in observed-to-expected analyses for vaccines. Pharmacoepidemiology and Drug Safety, 25(2), 215–222. https://doi.org/10.1002/pds.3918

Marin, M., Marti, M., Kambhampati, A., Jeram, S. M., & Seward, J. F. (2016). Global Varicella Vaccine Effectiveness: A Meta-analysis. Pediatrics, 137(3). https://doi.org/10.1542/peds.2015-3741

Marshall, H. S., McMillan, M., Koehler, A. P., Lawrence, A., Sullivan, T. R., MacLennan, J. M., et al. (2020). Meningococcal B Vaccine and Meningococcal Carriage in Adolescents in Australia. New England Journal of Medicine. https://doi.org/10.1056/NEJMoa1900236

Martín Arias, L. H., Sáinz, M., Treceño, C., & Carvajal, A. (2015). Guillain-Barré syndrome and influenza vaccines: A meta-analysis. Vaccine, 33(31), 3773–3778. https://doi.org/10.1016/j.vaccine.2015.05.013

McClure, D. L., Glanz, J. M., & Xu, S. (2008). Comparison of epidemiologic methods for active surveillance of vaccine safety. Vaccine, 26(26), 3341–3345. https://doi.org/10.1016/j.vaccine.2008.03.074

Menni, C., Klaser, K., May, A., Polidori, L., Capdevila, J., Louca, P., et al. (2021). Vaccine side-effects and SARS-CoV-2 infection after vaccination in users of the COVID Symptom Study app in the UK: a prospective observational study. The Lancet Infectious Diseases, 0(0). https://doi.org/10.1016/S1473-3099(21)00224-3

Miller, E., Andrews, N., Stellitano, L., Stowe, J., Winstone, A. M., Shneerson, J., & Verity, C. (2013). Risk of narcolepsy in children and young people receiving AS03 adjuvanted pandemic A/H1N1 2009 influenza vaccine: retrospective analysis. BMJ, 346. https://doi.org/10.1136/bmj.f794

Milman, O., Yelin, I., Aharony, N., Katz, R., Herzel, E., Ben-Tov, A., et al. (2021). Community-level evidence for SARS-CoV-2 vaccine protection of unvaccinated individuals. Nature Medicine, 1–3. https://doi.org/10.1038/s41591-021-01407-5

MODULE 4 – Overview and outcomes - WHO Vaccine Safety Basics. https://vaccine-safety-training.org/overview-and-outcomes-4.html Accessed 17 June 2021

Murray, J., & Cohen, A. L. (2017). Infectious Disease Surveillance. International Encyclopedia of Public Health, 222–229. https://doi.org/10.1016/B978-0-12-803678-5.00517-8

Nelson, J. C., Cook, A. J., Yu, O., Dominguez, C., Zhao, S., Greene, S. K., et al. (2012). Challenges in the design and analysis of sequentially monitored postmarket safety surveillance evaluations using electronic observational health care data. Pharmacoepidemiology and Drug Safety, 21(S1), 62–71. https://doi.org/10.1002/pds.2324

New Self-Controlled Case Series Method for Analyzing Spontaneous Reports of Adverse Events After Vaccination | American Journal of Epidemiology | Oxford Academic. https://academic.oup.com/aje/article/178/9/1496/89072

Omersel, J., & Karas Kuželički, N. (2020). Vaccinomics and Adversomics in the Era of Precision Medicine: A Review Based on HBV, MMR, HPV, and COVID-19 Vaccines. Journal of Clinical Medicine, 9(11), 3561. https://doi.org/10.3390/jcm9113561

Patel, M. M., Jackson, M. L., & Ferdinands, J. (2020). Postlicensure Evaluation of COVID-19 Vaccines. JAMA, 324(19), 1939. https://doi.org/10.1001/jama.2020.19328

Pebody, R. (2012). Vaccine registers – experiences from Europe and elsewhere. Eurosurveillance, 17(17), 20159. https://doi.org/10.2807/ese.17.17.20159-en

Polyzos, K. A., Konstantelias, A. A., Pitsa, C. E., & Falagas, M. E. (2015). Maternal Influenza Vaccination and Risk for Congenital Malformations: A Systematic Review and Meta-analysis. Obstetrics & Gynecology, 126(5), 1075–1084. https://doi.org/10.1097/AOG.0000000000001068

Pottegård, A., Lund, L. C., Karlstad, Ø., Dahl, J., Andersen, M., Hallas, J., et al. (2021). Arterial events, venous thromboembolism, thrombocytopenia, and bleeding after vaccination with Oxford-AstraZeneca ChAdOx1-S in Denmark and Norway: population based cohort study. BMJ, 373. https://doi.org/10.1136/bmj.n1114

Protocol_for_Cluster_Investigations_to_Measure_Influenza_Vaccine_Effectiveness - ECDC. (n.d.). https://www.ecdc.europa.eu/sites/default/files/media/en/publications/Publications/0912_TED_Protocol_for_Cluster_Investigations_to_Measure_Influenza_Vaccine_Effectiveness.pdf

Rapid assessment of COVID-19 vaccines safety concerns through electronic health records: a protocol template from the ACCESS project - ACCESS. (n.d.). https://vac4eu.org/wp-content/uploads/2021/02/3b.Rapid-assessment-of-COVID-19-vaccines-safety-concerns-through-electronic-health-records-a-protocol-template-from-the-ACCESS-project-.pdf Accessed 21 June 2021

Rasmussen, T. A., Jørgensen, M. R. S., Bjerrum, S., Jensen-Fangel, S., Støvring, H., Østergaard, L., & Søgaard, O. S. (2012). Use of population based background rates of disease to assess vaccine safety in childhood and mass immunisation in Denmark: nationwide population based cohort study. The BMJ, 345. https://doi.org/10.1136/bmj.e5823

Remschmidt, C., Rieck, T., Bödeker, B., & Wichmann, O. (2015). Application of the screening method to monitor influenza vaccine effectiveness among the elderly in Germany. BMC Infectious Diseases, 15(1), 137. https://doi.org/10.1186/s12879-015-0882-3

Renoud, L., Khouri, C., Revol, B., Lepelley, M., Perez, J., Roustit, M., & Cracowski, J.-L. (2021). Association of Facial Paralysis With mRNA COVID-19 Vaccines: A Disproportionality Analysis Using the World Health Organization Pharmacovigilance Database. JAMA Internal Medicine. https://doi.org/10.1001/jamainternmed.2021.2219

Report of the CIOMS/WHO Working Group on Definition and Application of Terms for Vaccine Pharmacovigilance.
https://www.who.int/vaccine_safety/initiative/tools/CIOMS_report_WG_vaccine.pdf

Rodriguez-Nava, G., Egoryan, G., Trelles-Garcia, D. P., Yanez-Bello, M. A., & Murguia-Fuentes, R. (2021). Disproportionality analysis of anaphylactic reactions after vaccination with messenger RNA coronavirus disease 2019 vaccines in the United States. Annals of Allergy, Asthma & Immunology, 0(0). https://doi.org/10.1016/j.anai.2021.04.004

Rondy, M., Issifou, D., Ibrahim, A. S., Maman, Z., Kadade, G., Omou, H., et al. (2016). Vaccine Effectiveness of Polysaccharide Vaccines Against Clinical Meningitis – Niamey, Niger, June 2015. PLOS Currents Outbreaks.
https://doi.org/10.1371/currents.outbreaks.5d6e9c1d071a2088109c242771b68886

Rondy, M., Launay, O., Puig-Barberà, J., Gefenaite, G., Castilla, J., Donati, K. de G., et al. (2015). 2012/13 influenza vaccine effectiveness against hospitalised influenza A(H1N1)pdm09, A(H3N2) and B: estimates from a European network of hospitals. Eurosurveillance, 20(2), 21011.
https://doi.org/10.2807/1560-7917.ES2015.20.2.21011

Rosillon, D., Willame, C., Tavares Da Silva, F., Guignard, A., Caterina, S., Welby, S., & Struyf, F. (2020). Meta-analysis of the risk of autoimmune thyroiditis, Guillain-Barré syndrome, and inflammatory bowel disease following vaccination with AS04-adjuvanted human papillomavirus 16/18 vaccine. Pharmacoepidemiology and Drug Safety, 29(9), 1159–1167.
https://doi.org/10.1002/pds.5063

Salmon, D. A., Proschan, M., Forshee, R., Gargiullo, P., Bleser, W., Burwen, D. R., et al. (2013). Association between Guillain-Barré syndrome and influenza A (H1N1) 2009 monovalent inactivated vaccines in the USA: a meta-analysis. The Lancet, 381(9876), 1461–1468.
https://doi.org/10.1016/S0140-6736(12)62189-8

Seabroke, S., Candore, G., Juhlin, K., Quarcoo, N., Wisniewski, A., Arani, R., et al. (2016). Performance of Stratified and Subgrouped Disproportionality Analyses in Spontaneous Databases. Drug Safety, 39(4), 355–364. https://doi.org/10.1007/s40264-015-0388-3

Simpson, C. R., Lone, N. I., Kavanagh, K., Ritchie, L. D., Robertson, C., Sheikh, A., & McMenamin, J. (2015). Trivalent inactivated seasonal influenza vaccine effectiveness for the prevention of laboratory-confirmed influenza in a Scottish population 2000 to 2009. Eurosurveillance, 20(8), 21043.
https://doi.org/10.2807/1560-7917.ES2015.20.8.21043

Smedt, T. D., Merrall, E., Macina, D., Perez-Vilar, S., Andrews, N., & Bollaerts, K. (2018). Bias due to differential and non-differential disease- and exposure misclassification in studies of vaccine effectiveness. PLOS ONE, 13(6), e0199180. https://doi.org/10.1371/journal.pone.0199180

Sullivan, S. G., Tchetgen, T., J, E., & Cowling, B. J. (2016). Theoretical Basis of the Test-Negative Study Design for Assessment of Influenza Vaccine Effectiveness. American Journal of Epidemiology, 184(5), 345–353. https://doi.org/10.1093/aje/kww064

Systematic overview of data sources for drug safety in pregnancy research - University of Bath.
http://www.encepp.eu/structure/documents/Data_sources_for_medicines_in_pregnancy_research.pdf

Tavares-Da-Silva, F., Miranda Co, M., & Dessart, C. (2020). Review of the initial post-marketing safety surveillance for the recombinant zoster vaccine. Vaccine, 38(18), 3489–3500.
https://doi.org/10.1016/j.vaccine.2019.11.058

Team, W. H. O. Q. A. and S. of M. (2006). The safety of medicines in public health programmes : pharmacovigilance, an essential tool. World Health Organization. https://apps.who.int/iris/handle/10665/43384

Théophile, H., Moore, N., Robinson, P., Bégaud, B., & Pariente, A. (2016). Vaccine Case–Population: A New Method for Vaccine Safety Surveillance. Drug Safety, 39(12), 1197–1209. https://doi.org/10.1007/s40264-016-0449-2

Tokars, J. I., Lewis, P., DeStefano, F., Wise, M., Viray, M., Morgan, O., et al. (2012). The Risk of Guillain–Barré Syndrome Associated with Influenza A (H1N1) 2009 Monovalent Vaccine and 2009–2010 Seasonal Influenza Vaccines: Results from Self-Controlled Analyses. Pharmacoepidemiology and Drug Safety, 21(5), 546–552. https://doi.org/10.1002/pds.3220

Vaccine Effectiveness Simulation. https://apps.p-95.com/VEMisclassification/

Vaccine Safety Datalink (VSD) | VSD | Monitoring | Ensuring Safety | Vaccine Safety | CDC. (2020, August 24). CDC. https://www.cdc.gov/vaccinesafety/ensuringsafety/monitoring/vsd/index.html

Van Holle, L., & Bauchau, V. (2013). Optimization of a quantitative signal detection algorithm for spontaneous reports of adverse events post immunization. Pharmacoepidemiology and Drug Safety, 22(5), 477–487. https://doi.org/10.1002/pds.3392

Vandenbroucke, J. P., & Pearce, N. (2019). Test-Negative Designs: Differences and Commonalities with Other Case–Control Studies with "Other Patient" Controls. Epidemiology, 30(6), 838–844. https://doi.org/10.1097/EDE.0000000000001088

Verani, J. R., Baqui, A. H., & Broome, C. V. (2017a). Case-control vaccine effectiveness studies: Data collection, analysis and reporting results. Vaccine, 35(25), 3303–3308. https://doi.org/10.1016/j.vaccine.2017.04.035

Verani, J. R., Baqui, A. H., & Broome, C. V. (2017b). Case-control vaccine effectiveness studies: Preparation, design, and enrollment of cases and controls. Vaccine, 35(25), 3295–3302. https://doi.org/10.1016/j.vaccine.2017.04.037

Wacholder, S., Chen, B. E., Wilcox, A., Macones, G., Gonzalez, P., Befano, B., et al. (2010). Risk of miscarriage with bivalent vaccine against human papillomavirus (HPV) types 16 and 18: pooled analysis of two randomised controlled trials. BMJ, 340. https://doi.org/10.1136/bmj.c712

Whitaker, J. A., Ovsyannikova, I. G., & Poland, G. A. (2015). Adversomics: a new paradigm for vaccine safety and design. Expert review of vaccines, 14(7), 935–947. https://doi.org/10.1586/14760584.2015.1038249

WHO Vaccine Safety Basics. (n.d.). WHO. https://vaccine-safety-training.org/home.html Accessed 17 June 2021

Wijnans, L., Dodd, C. N., Weibel, D., & Sturkenboom, M. (2017). Bell's palsy and influenza(H1N1)pdm09 containing vaccines: A self-controlled case series. PLOS ONE, 12(5), e0175539. https://doi.org/10.1371/journal.pone.0175539

Willame, C., Dodd, C., Aa, L. van der, Picelli, G., Emborg, H.-D., Kahlert, J., et al. (2021). Incidence Rates of Autoimmune Diseases in European Healthcare Databases: A Contribution of the ADVANCE Project. Drug Safety, 44(3), 383–395. https://doi.org/10.1007/s40264-020-01031-1

Wiyeh, A. B., Abdullahi, L. H., Wonkam, A., Wiysonge, C. S., & Kaba, M. (2018). Effects of vaccines in patients with sickle cell disease: a systematic review protocol. BMJ Open, 8(3), e021140. https://doi.org/10.1136/bmjopen-2017-021140

Woo, E. J., Ball, R., Burwen, D. R., & Braun, M. M. (2008). Effects of Stratification on Data Mining in the US Vaccine Adverse Event Reporting System (VAERS). Drug Safety, 31(8), 667–674. https://doi.org/10.2165/00002018-200831080-00003

World Health. (2017). Evaluation of influenza vaccine effectiveness: a guide to the design and interpretation of observational studies. World Health Organization. https://apps.who.int/iris/handle/10665/255203

Xu, R., Luo, Y., & Chambers, C. (2012). Assessing the effect of vaccine on spontaneous abortion using time-dependent covariates Cox models. Pharmacoepidemiology and Drug Safety, 21(8), 844–850. https://doi.org/10.1002/pds.3301

Yanni, E. A., Ferreira, G., Guennec, M., El Hahi, Y., El Ghachi, A., Haguinet, F., et al. (2018). Burden of herpes zoster in 16 selected immunocompromised populations in England: a cohort study in the Clinical Practice Research Datalink 2000–2012. BMJ Open, 8(6). https://doi.org/10.1136/bmjopen-2017-020528

Yih, W. K., Kulldorff, M., Dashevsky, I., & Maro, J. C. (n.d.). A Broad Safety Assessment of the 9-Valent Human Papillomavirus Vaccine. American Journal of Epidemiology. https://doi.org/10.1093/aje/kwab022

Yih, W. K., Kulldorff, M., Fireman, B. H., Shui, I. M., Lewis, E. M., Klein, N. P., et al. (2011). Active Surveillance for Adverse Events: The Experience of the Vaccine Safety Datalink Project. Pediatrics, 127(Supplement 1), S54–S64. https://doi.org/10.1542/peds.2010-1722I

Young-Xu, Y., Thornton Snider, J., & Robertus, van A. (2019). Analysis of relative effectiveness of high-dose versus standard-dose influenza vaccines using an instrumental variable method. Vaccine, 37(11), 1484–1490. https://doi.org/10.1016/j.vaccine.2019.01.063

Zacay, G., Shasha, D., Bareket, R., Kadim, I., Hershkowitz Sikron, F., Tsamir, J., et al. (n.d.). BNT162b2 vaccine effectiveness in preventing asymptomatic infection with SARS-CoV-2 virus: a nationwide historical cohort study. Open Forum Infectious Diseases. https://doi.org/10.1093/ofid/ofab262

14.3. Design, implementation and analysis of pharmacogenomic studies

Alshabeeb, M. A., Deneer, V. H. M., Khan, A., & Asselbergs, F. W. (2019). Use of Pharmacogenetic Drugs by the Dutch Population. Frontiers in Genetics, 10. https://doi.org/10.3389/fgene.2019.00567

Benjamin, J. G., & Marylyn, D. R. (2011). Statistical Optimization of Pharmacogenomics Association Studies: Key Considerations from Study Design to Analysis. Current Pharmacogenomics and Personalized Medicine. https://www.eurekaselect.com/95656/article

Bozkurt, Ö., Verschuren, W. M. M., Wijer, B. M. A. van W., Knol, M. J., Boer, A. de, Grobbee, D. E., et al. (2008). Genetic variation in the renin-angiotensin system modifies the beneficial effects of ACE inhibitors on the risk of diabetes mellitus among hypertensives. Journal of Human Hypertension, 22(11), 774–780. https://doi.org/10.1038/jhh.2008.62

Carleton, B., Poole, R., Smith, M., Leeder, J., Ghannadan, R., Ross, C., et al. (2009). Adverse drug reaction active surveillance: developing a national network in Canada's children's hospitals. Pharmacoepidemiology and Drug Safety, 18(8), 713–721. https://doi.org/10.1002/pds.1772

Carr, D. F., O'Meara, H., Jorgensen, A. L., Campbell, J., Hobbs, M., McCann, G., et al. (2013). SLCO1B1 Genetic Variant Associated With Statin-Induced Myopathy: A Proof-of-Concept Study Using the Clinical Practice Research Datalink. Clinical Pharmacology & Therapeutics, 94(6), 695–701. https://doi.org/10.1038/clpt.2013.161

Cavallari, L., Beitelshees, A., Blake, K., Dressler, L., Duarte, J., Elsey, A., et al. (2017). The IGNITE Pharmacogenetics Working Group: An Opportunity for Building Evidence with Pharmacogenetic Implementation in a Real-World Setting. Clinical and Translational Science, 10(3), 143–146. https://doi.org/10.1111/cts.12456

Chambliss, A. B., & Chan, D. W. (2016). Precision medicine: from pharmacogenomics to pharmacoproteomics. Clinical Proteomics, 13(1), 25. https://doi.org/10.1186/s12014-016-9127-8

Chan, H. T., Chin, Y. M., & Low, S.-K. (2019). The Roles of Common Variation and Somatic Mutation in Cancer Pharmacogenomics. Oncology and Therapy, 7(1), 1–32. https://doi.org/10.1007/s40487-018-0090-6

Chaplin, M., Kirkham, J. J., Dwan, K., Sloan, D. J., Davies, G., & Jorgensen, A. L. (2020). STrengthening the Reporting Of Pharmacogenetic Studies: Development of the STROPS guideline. PLOS Medicine, 17(9), e1003344. https://doi.org/10.1371/journal.pmed.1003344

Chen, P., Lin, J.-J., Lu, C.-S., Ong, C.-T., Hsieh, P. F., Yang, C.-C., et al. (2011). Carbamazepine-Induced Toxic Effects and HLA-B*1502 Screening in Taiwan. New England Journal of Medicine, 364(12), 1126–1133. https://doi.org/10.1056/NEJMoa1009717

Clinical Pharmacogenetics Implementation Consortium. https://cpicpgx.org/ Accessed 22 June 2021

CPNDS | The Canadian Pharmacogenomics Network for Drug Safety. https://cpnds.ubc.ca/ Accessed 22 June 2021

Dennis, J., Hawken, S., Krewski, D., Birkett, N., Gheorghe, M., Frei, J., et al. (2011). Bias in the case-only design applied to studies of gene–environment and gene–gene interaction: a systematic review and meta-analysis. International Journal of Epidemiology, 40(5), 1329–1341. https://doi.org/10.1093/ije/dyr088

DIMITROVA, E. K. (2020, April 30). EMA recommendations on DPD testing prior to treatment with fluorouracil, capecitabine, tegafur and flucytosine. European Medicines Agency. Text. https://www.ema.europa.eu/en/news/ema-recommendations-dpd-testing-prior-treatment-fluorouracil-capecitabine-tegafur-flucytosine

Drews, J. (2000). Drug Discovery: A Historical Perspective. Science, 287(5460), 1960–1964. https://doi.org/10.1126/science.287.5460.1960

Duconge, J., & Ruaño, G. (2018). Preventing the exacerbation of health disparities by iatrogenic pharmacogenomic applications: lessons from warfarin. Pharmacogenomics, 19(11), 875–881. https://doi.org/10.2217/pgs-2018-0055

Ehmann, F., Caneva, L., Prasad, K., Paulmichl, M., Maliepaard, M., Llerena, A., et al. (2015). Pharmacogenomic information in drug labels: European Medicines Agency perspective. The Pharmacogenomics Journal, 15(3), 201–210. https://doi.org/10.1038/tpj.2014.86

Evans, D. M., & Davey Smith, G. (2015). Mendelian Randomization: New Applications in the Coming Age of Hypothesis-Free Causality. Annual Review of Genomics and Human Genetics, 16(1), 327–350. https://doi.org/10.1146/annurev-genom-090314-050016

Evans, W. E., & Relling, M. V. (1999). Pharmacogenomics: Translating Functional Genomics into Rational Therapeutics. Science, 286(5439), 487–491. https://doi.org/10.1126/science.286.5439.487

Evans, W. E., & Relling, M. V. (2004). Moving towards individualized medicine with pharmacogenomics. Nature, 429(6990), 464–468. https://doi.org/10.1038/nature02626

Gatto, N. M., Campbell, U. B., Rundle, A. G., & Ahsan, H. (2004). Further development of the case-only design for assessing gene–environment interaction: evaluation of and adjustment for bias. International Journal of Epidemiology, 33(5), 1014–1024. https://doi.org/10.1093/ije/dyh306

Hayney, M. S. (2002). Pharmacogenomics and infectious diseases: Impact on drug response and applications to disease management. American Journal of Health-System Pharmacy, 59(17), 1626–1631. https://doi.org/10.1093/ajhp/59.17.1626

Hellwege, J., Keaton, J., Giri, A., Gao, X., Velez Edwards, D. R., & Edwards, T. L. (2017). Population Stratification in Genetic Association Studies. Current protocols in human genetics, 95, 1.22.1-1.22.23. https://doi.org/10.1002/cphg.48

Hingorani, A. D., Windt, D. A. van der, Riley, R. D., Abrams, K., Moons, K. G. M., Steyerberg, E. W., et al. (2013). Prognosis research strategy (PROGRESS) 4: Stratified medicine research. BMJ, 346. https://doi.org/10.1136/bmj.e5793

Implementation of a multidisciplinary pharmacogenomics clinic in a community health system | American Journal of Health-System Pharmacy | Oxford Academic. https://academic.oup.com/ajhp/article/73/23/1956/5102145 Accessed 22 June 2021

Jorgensen, A. L., & Williamson, P. R. (2008). Methodological quality of pharmacogenetic studies: Issues of concern. Statistics in Medicine, 27(30), 6547–6569. https://doi.org/10.1002/sim.3420

Khoury, M. J., & Flanders, W. D. (1996). Nontraditional Epidemiologic Approaches in the Analysis of Gene Environment Interaction: Case-Control Studies with No Controls! American Journal of Epidemiology, 144(3), 207–213. https://doi.org/10.1093/oxfordjournals.aje.a008915

Leitsalu, L., Haller, T., Esko, T., Tammesoo, M.-L., Alavere, H., Snieder, H., et al. (2015). Cohort Profile: Estonian Biobank of the Estonian Genome Center, University of Tartu. International Journal of Epidemiology, 44(4), 1137–1147. https://doi.org/10.1093/ije/dyt268

Liu, Y., Nyunoya, T., Leng, S., Belinsky, S. A., Tesfaigzi, Y., & Bruse, S. (2013). Softwares and methods for estimating genetic ancestry in human populations. Human Genomics, 7(1), 1. https://doi.org/10.1186/1479-7364-7-1

Mallal, S., Phillips, E., Carosi, G., Molina, J.-M., Workman, C., Tomažič, J., et al. (2008). HLA-B*5701 Screening for Hypersensitivity to Abacavir. New England Journal of Medicine, 358(6), 568–579. https://doi.org/10.1056/NEJMoa0706135

McInnes, G., & Altman, R. B. (2020). Drug Response Pharmacogenetics for 200,000 UK Biobank Participants. In Biocomputing 2021 (Vols. 1-0, pp. 184–195). WORLD SCIENTIFIC. https://doi.org/10.1142/9789811232701_0018

McInnes, G., Lavertu, A., Sangkuhl, K., Klein, T. E., Whirl-Carrillo, M., & Altman, R. B. (2021). Pharmacogenetics at Scale: An Analysis of the UK Biobank. Clinical Pharmacology & Therapeutics, 109(6), 1528–1537. https://doi.org/10.1002/cpt.2122

Molokhia, M., & McKeigue, P. (2006). EUDRAGENE: European collaboration to establish a case–control DNA collection for studying the genetic basis of adverse drug reactions. Pharmacogenomics, 7(4), 633–638. https://doi.org/10.2217/14622416.7.4.633

Pereira, N. L., Farkouh, M. E., So, D., Lennon, R., Geller, N., Mathew, V., et al. (2020). Effect of Genotype-Guided Oral P2Y12 Inhibitor Selection vs Conventional Clopidogrel Therapy on Ischemic Outcomes After Percutaneous Coronary Intervention: The TAILOR-PCI Randomized Clinical Trial. JAMA, 324(8), 761. https://doi.org/10.1001/jama.2020.12443

Peters, B. J. M., Rodin, A. S., De Boer, A., & Maitland-van der Zee, A.-H. (2010). Methodological and statistical issues in pharmacogenomics. Journal of Pharmacy and Pharmacology, 62(2), 161–166. https://doi.org/10.1211/jpp.62.02.0002

Peterson, J. F., Field, J. R., Shi, Y., Schildcrout, J. S., Denny, J. C., McGregor, T. L., et al. (2016). Attitudes of clinicians following large-scale pharmacogenomics implementation. The Pharmacogenomics Journal, 16(4), 393–398. https://doi.org/10.1038/tpj.2015.57

PharmGKB. (n.d.). PharmGKB. https://www.pharmgkb.org/

Pharmacogenomics Working Party. European Medicines Agency. https://www.ema.europa.eu/en/committees/working-parties-other-groups/chmp/pharmacogenomics-working-party

Pritchard, J. K., Stephens, M., Rosenberg, N. A., & Donnelly, P. (2000). Association Mapping in Structured Populations. The American Journal of Human Genetics, 67(1), 170–181. https://doi.org/10.1086/302959

Psaty, B. M. (2002). Diuretic Therapy, the α-Adducin Gene Variant, and the Risk of Myocardial Infarction or Stroke in Persons With Treated Hypertension. JAMA, 287(13), 1680. https://doi.org/10.1001/jama.287.13.1680

Psaty, B. M., & Sitlani, C. (2013). The Cohorts for Heart and Aging Research in Genomic Epidemiology (CHARGE) Consortium as a Model of Collaborative Science. Epidemiology, 24(3), 346–348. https://doi.org/10.1097/EDE.0b013e31828b2cbb

Rafi, I., Crinson, I., Dawes, M., Rafi, D., Pirmohamed, M., & Walter, F. M. (2020). The implementation of pharmacogenomics into UK general practice: a qualitative study exploring barriers, challenges and opportunities. Journal of Community Genetics, 11(3), 269–277. https://doi.org/10.1007/s12687-020-00468-2

Reisberg, S., Krebs, K., Lepamets, M., Kals, M., Mägi, R., Metsalu, K., et al. (2019). Translating genotype data of 44,000 biobank participants into clinical pharmacogenetic recommendations: challenges and solutions. Genetics in Medicine, 21(6), 1345–1354. https://doi.org/10.1038/s41436-018-0337-5

Robarge, J. D., Li, L., Desta, Z., Nguyen, A., & Flockhart, D. A. (2007). The Star-Allele Nomenclature: Retooling for Translational Genomics. Clinical Pharmacology & Therapeutics, 82(3), 244–248. https://doi.org/10.1038/sj.clpt.6100284

Ross, S., Anand, S. S., Joseph, P., & Paré, G. (2012). Promises and challenges of pharmacogenetics: an overview of study design, methodological and statistical issues. JRSM Cardiovascular Disease, 1(1), 1–13. https://doi.org/10.1258/cvd.2012.012001

Sissung, T. M., McKeeby, J. W., Patel, J., Lertora, J. J., Kumar, P., Flegel, W. A., et al. (2017). Pharmacogenomics Implementation at the National Institutes of Health Clinical Center. The Journal of Clinical Pharmacology, 57(S10), S67–S77. https://doi.org/10.1002/jcph.993

Smit, R. a. j., Noordam, R., le Cessie, S., Trompet, S., & Jukema, J. w. (2018). A critical appraisal of pharmacogenetic inference. Clinical Genetics, 93(3), 498–507. https://doi.org/10.1111/cge.13178

Storey, J. D., & Tibshirani, R. (2003). Statistical significance for genomewide studies. Proceedings of the National Academy of Sciences, 100(16), 9440–9445.

Sudlow, C., Gallacher, J., Allen, N., Beral, V., Burton, P., Danesh, J., et al. (2015). UK Biobank: An Open Access Resource for Identifying the Causes of a Wide Range of Complex Diseases of Middle and Old Age. PLOS Medicine, 12(3), e1001779. https://doi.org/10.1371/journal.pmed.1001779

Swen, J., Nijenhuis, M., de Boer, A., Grandia, L., Maitland-van der Zee, A., Mulder, H., et al. (2011). Pharmacogenetics: From Bench to Byte— An Update of Guidelines. Clinical Pharmacology & Therapeutics, 89(5), 662–673. https://doi.org/10.1038/clpt.2011.34

Taylor, C., Crosby, I., Yip, V., Maguire, P., Pirmohamed, M., & Turner, R. M. (2020). A Review of the Important Role of CYP2D6 in Pharmacogenomics. Genes, 11(11). https://doi.org/10.3390/genes11111295

Thorn, C. F., Whirl-Carrillo, M., Hachad, H., Johnson, J. A., McDonagh, E. M., Ratain, M. J., et al. (2019). Essential Characteristics of Pharmacogenomics Study Publications. Clinical Pharmacology & Therapeutics, 105(1), 86–91. https://doi.org/10.1002/cpt.1279

van der Baan, F. H., Klungel, O. H., Egberts, A. C., Leufkens, H. G., Grobbee, D. E., Roes, K. C., & Knol, M. J. (2011). Pharmacogenetics in randomized controlled trials: considerations for trial design. Pharmacogenomics, 12(10), 1485–1492. https://doi.org/10.2217/pgs.11.95

van der Baan, F. H., Knol, M. J., Klungel, O. H., Egberts, A. C., Grobbee, D. E., & Roes, K. C. (2012). Potential of adaptive clinical trial designs in pharmacogenetic research. Pharmacogenomics, 13(5), 571–578. https://doi.org/10.2217/pgs.12.10

van der Wouden, C., Cambon-Thomsen, A., Cecchin, E., Cheung, K., Dávila-Fajardo, C., Deneer, V., et al. (2017). Implementing Pharmacogenomics in Europe: Design and Implementation Strategy of the Ubiquitous Pharmacogenomics Consortium. Clinical Pharmacology & Therapeutics, 101(3), 341–358. https://doi.org/10.1002/cpt.602

van Wieren-de Wijer, D. B., Maitland-van der Zee, A.-H., de Boer, A., Kroon, A. A., de Leeuw, P. W., Schiffers, P., et al. (2009). Interaction between the Gly460Trp α-adducin gene variant and diuretics on the risk of myocardial infarction. Journal of Hypertension, 27(1), 61–68. https://doi.org/10.1097/HJH.0b013e328317a74d

Zhang, H., Liu, L., Wang, X., & Gruen, J. (2007). Guideline for data analysis of genomewide association studies. Cancer genomics & proteomics. https://pubmed.ncbi.nlm.nih.gov/17726238/

14.4. Methods for pharmacovigilance impact research

Banerjee, A. K., Zomerdijk, I. M., Wooder, S., Ingate, S., & Mayall, S. J. (2014). Post-Approval Evaluation of Effectiveness of Risk Minimisation: Methods, Challenges and Interpretation. Drug Safety, 37(1), 33–42. https://doi.org/10.1007/s40264-013-0126-7

Bernal, J. L., Cummins, S., & Gasparrini, A. (2017). Interrupted time series regression for the evaluation of public health interventions: a tutorial. International Journal of Epidemiology, 46(1), 348–355. https://doi.org/10.1093/ije/dyw098

Bradley, C. J., Penberthy, L., Devers, K. J., & Holden, D. J. (2010). Health Services Research and Data Linkages: Issues, Methods, and Directions for the Future. Health Services Research, 45(5p2), 1468–1488. https://doi.org/10.1111/j.1475-6773.2010.01142.x

Cohen, A., Rabbani, A., Shah, N., & Alexander, G. C. (2010). Changes in Glitazone Use Among Office-Based Physicians in the U.S., 2003–2009. Diabetes Care, 33(4), 823–825. https://doi.org/10.2337/dc09-1834

Crijns, H. J. M. J., van Rein, N., Gispen-de Wied, C. C., Straus, S. M., & de Jong-van den Berg, L. T. W. (2012). Prescriptive contraceptive use among isotretinoin users in the Netherlands in comparison with non-users: a drug utilisation study. Pharmacoepidemiology and Drug Safety, 21(10), 1060–1066. https://doi.org/10.1002/pds.3200

Crijns, I., Mantel-Teeuwisse, A., Bloemberg, R., Pinas, E., Straus, S., & Berg, L. de J. den. (2013). Healthcare professional surveys to investigate the implementation of the isotretinoin Pregnancy Prevention Programme: a descriptive study. Expert Opinion on Drug Safety, 12(1), 29–38. https://doi.org/10.1517/14740338.2013.745850

Durbin, J., & Watson, G. S. (1950). Testing for Serial Correlation in Least Squares Regression: I. Biometrika, 37(3/4), 409–428. https://doi.org/10.2307/2332391

Durbin, J., & Watson, G. S. (1951). Testing for Serial Correlation in Least Squares Regression. II. Biometrika, 38(1/2), 159–177. https://doi.org/10.2307/2332325

Eccles, M., Grimshaw, J., Campbell, M., & Ramsay, C. (2003). Research designs for studies evaluating the effectiveness of change and improvement strategies. BMJ Quality & Safety, 12(1), 47–52. https://doi.org/10.1136/qhc.12.1.47

Eworuke, E., Lee, J.-Y., Soule, L., Popat, V., & Moeny, D. G. (2017). The impact of the boxed warning on the duration of use for depot medroxprogesterone acetate. Pharmacoepidemiology and Drug Safety, 26(7), 827–836. https://doi.org/10.1002/pds.4227

Feldstein, A. C., Smith, D. H., Perrin, N., Yang, X., Rix, M., Raebel, M. A., et al. (2006). Improved Therapeutic Monitoring With Several Interventions: A Randomized Trial. Archives of Internal Medicine, 166(17), 1848. https://doi.org/10.1001/archinte.166.17.1848

Goedecke, T., Morales, D. R., Pacurariu, A., & Kurz, X. (2018). Measuring the impact of medicines regulatory interventions – Systematic review and methodological considerations. British Journal of Clinical Pharmacology, 84(3), 419–433. https://doi.org/10.1111/bcp.13469

Good pharmacovigilance practices. European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/post-authorisation/pharmacovigilance/good-pharmacovigilance-practices Accessed 23 June 2021

Haneuse, S., & Daniels, M. (2016). A General Framework for Considering Selection Bias in EHR-Based Studies: What Data are Observed and Why? eGEMs (Generating Evidence & Methods to improve patient outcomes), 4(1). https://doi.org/10.13063/2327-9214.1203

Hawton, K., Bergen, H., Simkin, S., Dodd, S., Pocock, P., Bernal, W., et al. (2013). Long term effect of reduced pack sizes of paracetamol on poisoning deaths and liver transplant activity in England and Wales: interrupted time series analyses. BMJ, 346. https://doi.org/10.1136/bmj.f403

Hedenmalm, K., Kurz, X., & Morales, D. (2019). Effect of withdrawal of fusafungine from the market on prescribing of antibiotics and other alternative treatments in Germany: a pharmacovigilance impact study. European Journal of Clinical Pharmacology, 75(7), 979–984. https://doi.org/10.1007/s00228-019-02650-z

Horton, D. B., Gerhard, T., Davidow, A., & Strom, B. L. (2017). Impact of the black triangle label on prescribing of new drugs in the United Kingdom: lessons for the United States at a time of deregulation. Pharmacoepidemiology and Drug Safety, 26(11), 1307–1313. https://doi.org/10.1002/pds.4304

IMI Work Package 5: Benefit –Risk Integration and Visual Representation - PROTECT. (n.d.). https://protectbenefitrisk.eu/documents/JuhaerietalBenefitRiskWave1CasestudyreportRimonabantOct2011.pdf Accessed 23 June 2021

Jordan, S., Gabe-Walters, M. E., Watkins, A., Humphreys, I., Newson, L., Snelgrove, S., & Dennis, M. S. (2015). Nurse-Led Medicines' Monitoring for Patients with Dementia in Care Homes: A Pragmatic Cohort Stepped Wedge Cluster Randomised Trial. PLOS ONE, 10(10), e0140203. https://doi.org/10.1371/journal.pone.0140203

Jordan, S., Morris, J. K., Davies, G. I., Tucker, D., Thayer, D. S., Luteijn, J. M., et al. (2016). Selective Serotonin Reuptake Inhibitor (SSRI) Antidepressants in Pregnancy and Congenital Anomalies: Analysis of Linked Databases in Wales, Norway and Funen, Denmark. PLOS ONE, 11(12), e0165122. https://doi.org/10.1371/journal.pone.0165122

Kendrick, T., Stuart, B., & Newell, C. (2015). Changes in rates of recorded depression in English primary care 2003–2013: Time trend analyses of effects of the economic recession, and the GP contract quality outcomes framework (QOF). Journal of Affective Disorders, 180, 68–78. https://doi.org/10.1016/j.jad.2015.03.040

Kim, H.-J., Fay, M. P., Feuer, E. J., & Midthune, D. N. (2000). Permutation tests for joinpoint regression with applications to cancer rates. Statistics in Medicine, 19(3), 335–351. https://doi.org/10.1002/(SICI)1097-0258(20000215)19:3<335::AID-SIM336>3.0.CO;2-Z

Madison, T., Donner, B., Mutter, R., Mingrino, R., & Alvaro, G. (2019). Effectiveness of Risk Minimization Measures to Prevent Pregnancy Exposure to Mycophenolate-Containing Medicines in Europe. Pharmaceutical Medicine, 33(5), 395–406. https://doi.org/10.1007/s40290-019-00304-0

Marston, L., Nazareth, I., Petersen, I., Walters, K., & Osborn, D. P. J. (2014). Prescribing of antipsychotics in UK primary care: a cohort study. BMJ Open, 4(12), e006135. https://doi.org/10.1136/bmjopen-2014-006135

Martin, R. M., Wheeler, B. W., Metcalfe, C., & Gunnell, D. (2010). What was the immediate impact on population health of the recent fall in hormone replacement therapy prescribing in England? Ecological study. Journal of Public Health, 32(4), 555–564. https://doi.org/10.1093/pubmed/fdq021

Mohammad, I., Korkis, B., & Garwood, C. L. (2017). Incorporating Comprehensive Management of Direct Oral Anticoagulants into Anticoagulation Clinics. Pharmacotherapy: The Journal of Human Pharmacology and Drug Therapy, 37(10), 1284–1297. https://doi.org/10.1002/phar.1991

Morales, D. R., Morant, S. V., MacDonald, T. M., Mackenzie, I. S., Doney, A. S. F., Mitchell, L., et al. (2020). Impact of EMA regulatory label changes on systemic diclofenac initiation, discontinuation, and switching to other pain medicines in Scotland, England, Denmark, and The Netherlands. Pharmacoepidemiology and Drug Safety, 29(3), 296–305. https://doi.org/10.1002/pds.4955

Morrato, E. H., Nicol, G. E., Maahs, D., Druss, B. G., Hartung, D. M., Valuck, R. J., et al. (2010). Metabolic Screening in Children Receiving Antipsychotic Drug Treatment. Archives of Pediatrics & Adolescent Medicine, 164(4). https://doi.org/10.1001/archpediatrics.2010.48

Nkeng, L., Cloutier, A.-M., Craig, C., Lelorier, J., & Moride, Y. (2012). Impact of Regulatory Guidances and Drug Regulation on Risk Minimization Interventions in Drug Safety. Drug Safety, 35(7), 535–546. https://doi.org/10.2165/11599720-000000000-00000

Nyeland, M. E., Laursen, M. V., & Callréus, T. (2017). Evaluating the effectiveness of risk minimisation measures: the application of a conceptual framework to Danish real-world dabigatran data. Pharmacoepidemiology and Drug Safety, 26(6), 607–614. https://doi.org/10.1002/pds.4203

Practical Approaches to Risk Minimisation for Medicinal Products: Report of CIOMS Working Group IX - COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES. (2014). CIOMS. https://cioms.ch/publications/product/practical-approaches-to-risk-minimisation-for-medicinal-products-report-of-cioms-working-group-ix/

Rubino, A., & Artime, E. (2017). A descriptive review of additional risk minimisation measures applied to EU centrally authorised medicines 2006-2015. Expert Opinion on Drug Safety, 16(8), 877–884. https://doi.org/10.1080/14740338.2017.1335303

Shi, L., Zhao, Y., & Szymanski, K. (2011). Impact of thiazolidinedione safety warnings on medication use patterns and glycemic control among veterans with diabetes mellitus. Journal of Diabetes and its Complications, 25(3), 143–150. https://doi.org/10.1016/j.jdiacomp.2010.06.003

Singh, R. R., & Nayak, R. (2016). Impact of FDA Black Box Warning on Psychotropic Drug Use in Noninstitutionalized Elderly Patients Diagnosed With Dementia: A Retrospective Study. Journal of Pharmacy Practice, 29(5), 495–502. https://doi.org/10.1177/0897190015579451

Sobel, R. E., Blackwell, W., Fram, D. M., & Bate, A. (2019). A Novel Approach to Visualize Risk Minimization Effectiveness: Peeping at the 2012 UK Proton Pump Inhibitor Label Change Using a Rapid Cycle Analysis Tool. Drug Safety, 42(11), 1365–1376. https://doi.org/10.1007/s40264-019-00853-y

Sultana, J., Fontana, A., Giorgianni, F., Pasqua, A., Cricelli, C., Spina, E., et al. (2016). The Effect of Safety Warnings on Antipsychotic Drug Prescribing in Elderly Persons with Dementia in the United Kingdom and Italy: A Population-Based Study. CNS Drugs, 30(11), 1097–1109. https://doi.org/10.1007/s40263-016-0366-z

Sultana, J., Fontana, A., Giorgianni, F., Tillati, S., Cricelli, C., Pasqua, A., et al. (2019a). Measuring the Effectiveness of Safety Warnings on the Risk of Stroke in Older Antipsychotic Users: A Nationwide Cohort Study in Two Large Electronic Medical Records Databases in the United Kingdom and Italy. Drug Safety, 42(12), 1471–1485. https://doi.org/10.1007/s40264-019-00860-z

Sultana, J., Fontana, A., Giorgianni, F., Tillati, S., Cricelli, C., Pasqua, A., et al. (2019b). Measuring the Effectiveness of Safety Warnings on the Risk of Stroke in Older Antipsychotic Users: A Nationwide Cohort Study in Two Large Electronic Medical Records Databases in the United Kingdom and Italy. Drug Safety, 42(12), 1471–1485. https://doi.org/10.1007/s40264-019-00860-z

THE INTERNATIONAL MARCÉ SOCIETY FOR PERINATAL MENTAL HEALTH BIENNIAL SCIENTIFIC CONFERENCE. (2015). Archives of Women's Mental Health, 18(2), 269–408. https://doi.org/10.1007/s00737-014-0488-6

The limitations of some European healthcare databases for monitoring the effectiveness of pregnancy prevention programmes as risk minimisation measures | SpringerLink. https://link.springer.com/article/10.1007%2Fs00228-017-2398-9

Thompson, C. A., Kurian, A. W., & Luft, H. S. (2015). Linking Electronic Health Records to Better Understand Breast Cancer Patient Pathways Within and Between Two Health Systems. eGEMs (Generating Evidence & Methods to improve patient outcomes), 3(1). https://doi.org/10.13063/2327-9214.1127

Torun, P., Heller, R. F., & Verma, A. (2009). Potential population impact of changes in heroin treatment and smoking prevalence rates: using Population Impact Measures. European Journal of Public Health, 19(1), 28–31. https://doi.org/10.1093/eurpub/ckn103

Valuck, R. J., Libby, A. M., Orton, H. D., Morrato, E. H., Allen, R., & Baldessarini, R. J. (2007). Spillover Effects on Treatment of Adult Depression in Primary Care After FDA Advisory on Risk of Pediatric

Suicidality With SSRIs. American Journal of Psychiatry, 164(8), 1198–1205. https://doi.org/10.1176/appi.ajp.2007.07010007

Van Ganse, E., Belhassen, M., Ginoux, M., Chrétien, E., Cornu, C., Ecoffey, C., & Aubrun, F. (2018). Use of analgesics in France, following dextropropoxyphene withdrawal. BMC Health Services Research, 18(1), 231. https://doi.org/10.1186/s12913-018-3058-1

van Hunsel, F., Gardarsdottir, H., de Boer, A., & Kant, A. (2019). Measuring the impact of pharmacovigilance activities, challenging but important. British Journal of Clinical Pharmacology, 85(10), 2235–2237. https://doi.org/10.1111/bcp.14042

Weatherburn, C. J., Guthrie, B., Dreischulte, T., & Morales, D. R. (2020). Impact of medicines regulatory risk communications in the UK on prescribing and clinical outcomes: Systematic review, time series analysis and meta-analysis. British Journal of Clinical Pharmacology, 86(4), 698–710. https://doi.org/10.1111/bcp.14104

Yarrington, M. E., Anderson, D. J., Ashley, E. D., Jones, T., Davis, A., Johnson, M., et al. (2019). Impact of FDA black box warning on fluoroquinolone and alternative antibiotic use in southeastern US hospitals. Infection Control & Hospital Epidemiology, 40(11), 1297–1300. https://doi.org/10.1017/ice.2019.247

Zipitis, C. S., Mughal, Z. M., & Clayton, P. E. (2016). Assessing the population impact of low rates of vitamin D supplementation on type 1 diabetes using a new statistical method. JRSM Open, 7(11), 2054270416653522. https://doi.org/10.1177/2054270416653522

Zomerdijk, I. M., Ruiter, R., Houweling, L. M. A., Herings, R. M. C., Sturkenboom, M. C. J. M., Straus, S. M. J. M., & Stricker, B. H. (2014). Isotretinoin exposure during pregnancy: a population-based study in The Netherlands. BMJ Open, 4(11), e005602. https://doi.org/10.1136/bmjopen-2014-005602