QUEST CENTER
Transforming Biomedical Research
Berlin Institute of Health

DLR-PT

Preclinical Confirmatory Studies and Systematic Reviews

Glossary

Term	Definition	Reference
Accessibility of results	The degree to which results are accessible e.g. for other researchers, see also Open Access and Open Data	-
Bias	Systematic error in an estimate (e.g., variance, effect size) caused by violations of assumptions, inadequacies in the design, conduct, or analysis of an experiment.	https://catalogofbias.org/
Blinding	Procedure to prevent performance bias. Will ensure that experimenters are unaware of the intervention condition during outcome assessment and analysis	https://catalogofbias.org/biases/performance-bias/
Comparator	Comparator group acts as a control with an effective intervention (as opposed to a placebo group). Allows comparison of effect sizes between two effective treatments.	Active comparator arm: https://clinicaltrials.gov/ct2/about-studies/glossary

Term	Definition	Reference
Confirmatory preclinical study	Experimental studies to scrutinize preclinical findings through replication of results alongside investigations into boundary conditions and robustness through conduct of additional (control) conditions and multi center studies. See also Exploratory preclinical study	https://doi.org/10.1371/journal.pbio.1001863
Construct Validity	Extent to which a measured outcome is representative of the phenomenon studied	doi: 10.1371/journal.pmed.1001489
Control (negative, positive control)	A baseline comparison group that receives no or placebo treatment (negative) or an effective comparison treatment (positive; see comparator).	-
Data management	Protocol describing data storage, data sharing, data preservation, and data description	http://www.dcc.ac.uk/webfm_send/1279
Data security	Models to ensure management of threats to data privacy, storage, longevity	-

Term	Definition	Reference
Data sharing	Making data accessible and reusable for others to different degrees by providing direct links to repositories or describing procedures through which data can be obtained.	-
Data synthesis strategy	Strategy to combine data from various sources into one estimate of an effect (e.g., strategy for a meta-analysis)	-
Design of a study	The setup of a study, specifically what interventions take place, what measurements are taken, what procedures are implemented to reduce bias	https://www.nc3rs.org.uk/experimental-design-assistant-eda
Dissemination of results	The way results are communicated and shared with peers and a broader public. See also Open Access	http://ec.europa.eu/research/participants/docs/h2020-funding-guide/grants/grant-management/dissemination-of-results_en.htm
Effect size	Quantitative measure that estimates the magnitude of differences between groups, or relationships between variables.	https://doi.org/10.4300/JGME-D-12-00156.1

Term	Definition	Reference
Efficacy of the intervention	Outcome measure of the effect of interest. See also Effect Size.	-
Experimental unit	Biological entity subjected to an intervention independently of all other units, such that it is possible to assign any two experimental units to different treatment groups.	https://eda.nc3rs.org.uk/experimental-design-unit
Exploratory preclinical study	Studies that search for novel biological associations and phenomena in preclinical settings often employing less rigorous experimental designs, low sample sizes. Further, many analyses are conducted to generate hypotheses. To avoid false positive results, discovered effects need to be confirmed in further preclinical studies	https://doi.org/10.1371/journal.pbio.1001863
Ecological validity	Extent to which results provide a correct basis for generalisations to other contexts of the same population.	Paragraph 2.6: https://learningstatisticswithr.com/book/studydesign.html
External Validity	Extent to which results provide a correct basis for generalisations to other populations or specifically in preclinical research to humans.	Paragraph 2.6: https://learningstatisticswithr.com/book/studydesign.html

Term	Definition	Reference
Extraction of data from primary studies	Process of extracting the relevant information to a synthesis from individual studies (e.g., an effect size to be included in a meta-analysis)	-
FAIR criteria	Acronym for data sharing standards: Findable, Accessible, Interoperable, Reusable.	http://www.nature.com/articles/d41586-019-01720-7
False positive	Statistically significant result obtained by chance when the effect being investigated does not exist.	https://doi.org/10.1038/nmeth.2738
False negative	Statistically non-significant result obtained when the effect being investigated genuinely exists.	https://doi.org/10.1038/nmeth.2738
Feasibility	Likelihood that an experiment can be conducted under the given constraints (number of experimental units available, laboratory equipement, time, personal)	

Term	Definition	Reference
Independent variable of interest	Factor that a researcher manipulates within a controlled environment in order to test its impact on the outcome measured. Also known as: predictor variable, factor of interest.	Paragraph 2.4: https://learningstatisticswithr.com/book/studydesign.html
Internal Validity	Refers to the rigour of the study design and statistical analysis to isolate cause and effect, and attribute the effect observed to manipulation of the independent variable of interest. In an experiment with high internal validity, sources of bias and chance observations are minimised. See Bias, Randomisation, and Blinding	Paragraph 2.6: https://learningstatisticswithr.com/book/studydesign.html
Long-term safeguarding of research data	Preservation of data over long time scales (at least 10 years). See also data security, data management	https://www.dfg.de/foerderung/antrag_gutachter_gremien/antragstellende/nachnutzung_forschungsdaten/index.html
Metadata	Data about experimental, datasets outlining content, timestamp, authors, explanation of variables.	https://doi.org/10.1038/sdata.2016.18
Methods against bias	Procedures to reduce bias in a study like randomisation and blinding but also improved statistical methods	-

Term	Definition	Reference
Multicenter study	Procedure where multiple labs collect data for the same study design to investigate variability between labs to estimate generalisability of results	https://journals.plos.org/plosbiology/article?id=10.1371/journal.pbio.2003693
Nuisance variable	Sources of variability or conditions that could potentially bias results. Also known as: confounding factor, confounding variable	Paragraph 2.7: https://learningstatisticswithr.com/book/studydesign.html
Null Hypothesis Significance Testing	NHST is a method of statistical inference to assess the probability that the observed data (or more extreme) will occur given a null hypothesis of no effect of our variable of interest.	https://www.nature.com/articles/nmeth.2698
Open access	Freely available research articles for anyone, no subscription needed.	https://doi.org/10.12688/f1000research.8460.3
Open data	Freely available and reusable data, without barriers see FAIR criteria	https://book.fosteropenscience.eu/en/02OpenScienceBasics/02OpenResearchDataAndMaterials.html

Term	Definition	Reference
Outcome measure	Any variable recorded during a study to assess the effects of a treatment or experimental intervention. Also known as: dependent variable, response variable	Paragraph 2.4: https://learningstatisticswithr.com/book/studydesign.html
Statistical Power	Probability that a significance test detects an effect (i.e. a deviation from the null hypothesis), if an effect exists (i.e. true positive result).	https://doi.org/10.1177/0956797616647519
Pre-registration of the study, pre-registration of study protocol	Formal registration of methods, experimental design, and analysis procedures prior to data-collection	https://doi.org/10.1177/1745691618771357
Registered Report	Type of article that undergoes a two-stage review. Before data collection reviewers assess the scientific premise and in depth methodology including an analysis plan. Upon acceptance in stage 1, articles are in principle accepted independent of the results. After data collection, reviewers only assess whether experiments were conducted according to the plan from stage 1.	https://doi.org/10.1016%2Fj.cortex.2012.12.016
Preprint	Sharing of a manuscript prior to peer-review mostly on dedicated repositories like BioArxiv.	https://www.plos.org/why-preprint

Term	Definition	Reference
Publication bias	Studies remain often unpublished when an outcome is contrary to the initial hypothesis. This leads to an overrepresentation of positive findings impeding a meta analytic assessment of the investigated effect.	https://catalogofbias.org/biases/publication-bias/
Quality management	Procedures in a laboratory to increase study quality through standard operating procedures that ensure rigorous experimental design and reduction of measurement errors.	https://doi.org/10.15252/embr.201847143
Quality-assessment of primary studies	Retrospective procedure to evaluate whether studies follow reporting standards and whether they are at a risk of bias.	https://handbook-5-1.cochrane.org/chapter_8/table_8_5_a_the_cochrane_collaborations_tool_for_assessing.htm
Randomisation	Assignment procedure to control for random variations between experimental units included in a study.	https://eda.nc3rs.org.uk/experimental-design-allocation
Repository	Online database where information like experimental data or analysis code is deposited and archived	-

Term	Definition	Reference
Rigor	The strict application of the scientific method to ensure robust and unbiased experimental design, methodology, analysis, interpretation and reporting of results. This includes full transparency in reporting experimental details so that others may reproduce and extend the findings.	https://grants.nih.gov/reproducibility/faqs.htm#4828
Sample size	Number of experimental units per group, also referred to as N number. See also Statistical Power and Sample Size determination	-
Sample size determination	Procedures to determine how many experimental units to include in a design. Depends on power, effect size, and significance level	https://doi.org/10.1038/nmeth.2738
Scientific premise	Rigor of the prior research: strengths and weakness of the data and previously performed work upon which a study is built upon	https://nexus.od.nih.gov/all/2016/01/28/scientific-premise-in-nih-grant-applications/
Search strategy	Strategy to search databases for relevant studies, typically done systematically with a pre-specified set of searches and search terms.	-

Term	Definition	Reference
Sharing of data	The extent to which data is made available to others, see also Open Data	-
Study quality	The assessment of a study's quality with respect to design, measurement, question	-
Triangulation	Scientific method for evidence generation through use of different approaches addressing the same underlying question under consideration of specific biases of each approach.	https://doi.org/10.1038/d41586-018-01023-3