



Annie Robert,

IREC - UCL

" Some pitfalls in the analysis and inference from clinical studies "

Clinical research has two major aims: (1) to increase diagnostic reliability of diseases by developing diagnostic tools and (2) to cure patients or, at least, to control disease evolution by learning from prognosis assessment. Both areas of clinical research are highly related because it's useless to have a very good diagnostic tool if there is no treatment and a very good treatment against a disease is useless if the disease can't be detected. In both areas, there are 3 basic principles behind clinical research: (1) Comparison, (2) Signification, and (3) Causality. Even with a clearly focused and relevant research question, pitfalls can arise in comparisons, in significations, and in causal inference, mainly because (1) clinical research deals with "Convenience samples" and statistics deal with "i.i.d. samples", (2) clinical differences and statistical significances are often confused (lack of sizes computations), and (3) randomization between groups for comparisons is rarely possible; Phase III clinical trials are less than 1% of medical research. Examples will be presented to illustrate some pitfalls. In diagnostic research area, the definition of "no disease" can mislead the comparisons and estimations and statistical significance should be questioned if statistical units are not independent. In prognostic research area, there is a big difference between randomized trials and observational studies to conclude that a relationship is one of cause and effect. Propensity scores (PS) will be presented as an additional tool available to investigators as they try to estimate the effects of treatments in studies where potential biases may exist. Use of propensity scores does not correct biases from unmeasured confounders and PS will never replace randomization but causality can find a place in observational studies if confounders are measured before treatment options and treatment allocation are decided before outcome measurement. Pitfalls and biases caused by missing data will not be presented because it's another long story that cannot be addressed without a solid knowledge of the specific clinical context.

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