

Kolloquium „Statistische Methoden in der empirischen Forschung“

Wann: 22. November 2016, 17:00 – 18:30 Uhr

Wo: Robert Koch-Institut | Nordufer 20 | 13353 Berlin (Wedding),
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Commissioning by evaluation for long term conditions – 10 years of the UK multiple sclerosis risk sharing scheme

Assessing the cost-effectiveness of treatments for long-term medical conditions such as multiple sclerosis (MS) poses enormous challenges. The real benefit of disease-modifying treatments (DMTs) may only become apparent 10 or more years into the course of the disease; yet we have to assess these benefits on the basis of evidence from randomised controlled trial of, typically, only 2-3 years' duration.

The UK MS risk sharing scheme was set up in 2002 to address precisely this problem in relation to the “first generation” DMTs for MS, three forms of interferon beta and glatiramer acetate. The UK's National Institute for Health and Clinical Effectiveness (NICE) had assessed the four drugs but had not found enough evidence of their long term benefits to justify the (then) high price. The risk sharing scheme was an agreement between the UK health departments, patient and professional groups, and the 4 companies to enable the drugs to be made available in the UK. The key element was a 10-year monitoring study of a cohort of some 5,500 patients, to assess whether the observed benefits of treatment were in line with those predicted on the basis of the pivotal RCTs of the drugs. If observed benefits fell short of predictions, the price of the drugs would be reduced to restore cost-effectiveness.

[The price adjustment mechanism therefore involves a comparison between the observed disability progression of patients in the monitoring cohort, and the progression that would have been expected on the basis of the treatment effects estimated in the RCTs. For this comparison we used two complementary models – a continuous time Markov model and a multi-level repeated measures model – and a wide array of sensitivity and subgroup analyses.]

The monitoring study has recently been completed and the results of the final analysis are [as of July 2016] being prepared for publication. In this colloquium, I will reflect both on the statistical challenges in analysing observational data of this kind, and on some of the more general and practical issues in maintaining a study of such length when the different parties have inevitably very different interests in the outcomes. “Commissioning by evaluation” is once again a fashionable concept, but sadly it is most difficult to achieve precisely in the circumstances in which it is most needed.