

MEDIASRES Publishable Summary

Final report: 1 January 2012 – 31 December 2015

Marie Curie Initial Training Network GA No. 290025



Scientific Coordinator: Prof. Martin Schumacher
Center for Medical Biometry and Medical Informatics
University of Freiburg, Germany
ms@imbi.uni-freiburg.de or mediasres@imbi.uni-freiburg.de

Webpage: www.mediasres-itn.eu

Clinical trials, diagnostic and prognostic studies are the primary source of evidence in clinical medicine as well as for developing new therapeutic and diagnostic modalities. Contributions over the last decades have led to state-of-the-art statistical methodology that is now considered standard for designing and analyzing such studies and for synthesis of their results by meta-analytic approaches. However, with the increasing availability of genomic and other molecular techniques, the road seems to be paved towards individualized diagnosis, prognosis, and treatment, usually referred to as personalized medicine. This leads to new challenges for the design and analysis of studies. The corresponding complex structures have to be modelled adequately and results have to be aggregated by corresponding advanced meta-analytic approaches. Novel statistical methodology promises to greatly facilitate development of approaches for personalized medicine.

The research area of statistical **ME**thodology for **DI**agnostic/prognostic and therapeutic **S**tudies and systematic **RE**views (**MEDIASRES**) is well suited for early-stage training purposes, as it comprises a wide range of theoretical and applied biostatistical tools that need to be mastered, further developed, and translated into clinical research and practice. MEDIASRES reached the main network goal in establishing a joint research-training programme by an interdisciplinary and intersectorial network (including 3 partners from the private sector) for providing 14 early-stage researchers (ESRs) with deep insight into cutting-edge statistical methodology in order to face current and future challenges in the design and statistical analysis of clinical research.

Work performed within the project

Scientific research

All 14 ESRs have worked extensively on their individual research projects. Major methodological research areas involved longitudinal data, time-to-event data, multistate models, meta-analysis, prediction, regression models for considering genomic or other molecular data, pharmacokinetic/pharmacodynamic modelling, adaptive design and dose finding, non-compliance, missing data and biased sampling. The scientific output has been and will be published in numerous journals (see Dissemination).

Training activities

MEDIASRES established a fruitful training environment for all ESRs. MEDIASRES provided the ESRs with the breadth and depth of analytical tools and conceptual knowledge needed to address the challenges of modern trial design and data analysis in biostatistics. In total, five face-to-face network-wide training weeks as well as two extensive online courses were conducted in addition to

numerous local courses and workshops. Training sessions included basic and specialized statistical courses from a wide range of current topics (epidemiology, meta-analysis, competing risks, Bayesian statistics, causal inference, repeated measurements, multistate models) and practicals (R, ggplot and orgmode) as well as transferable skills courses (such as good scientific practice, scientific, reading, writing and presentation, grant writing, project management and career planning). Transnational research secondments were an essential part of MEDIASRES and complete the picture. Each ESR spent at least 2 months at a Partner Institution whenever possible from academia to industry and vice versa to gain insight into different working environments.

One ESR has already obtained her Ph.D., all others are close to submitting or have already submitted their Ph.D. thesis' and are expected to obtain their Ph.D.s in the first half of 2016.

Dissemination

- 12 peer-reviewed papers published as per February 25th , 2016. Numerous results are currently submitted or in preparation for publication (> 15 papers).
- Results were presented by ESRs at numerous international conferences (> 65 talks and posters).
- A symposium with 150 participants was organized in conjunction with the final MEDIASRES Course Week.
- The MEDIASRES website is maintained and frequently updated.

Deliverables and Milestones

All deliverables and milestones of the project were successfully completed.

Final results and their potential impact and use

The MEDIASRES project contributed substantially to the development of novel methodology for current research questions investigated in diagnostic/prognostic and therapeutic studies. One particular strength of the project is that statistical design and modelling approaches are simultaneously considered on both study and meta-analysis level helping to generalize achieved results. A second particular strength is that the supervisors are internationally recognized biostatisticians from various academic institutions as well as from industry assuring that statistical approaches developed are based on theoretically sound statistical methodology and are fit and well-suited for practical application. The contributions from the MEDIASRES project have received considerable attention on international conferences and in top biostatistical journals. The additional top-level training activities will ensure that fourteen optimally trained young biostatisticians will join the quest for answers to the burning questions in biomedical research.