Ph.D. in Data Analytics and Decision Sciences

Thesis Defense

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Ph.D. candidate: Federica CORSO **- XXXVI Cycle**

A REAL-WORLD DATA STATISTICAL APPROACH TO SUPPORT DECISION-MAKING IN CLINICAL AND HEALTH POLICY

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Abstract:

Real-world evidence can complement randomized clinical trial findings by investigating the effectiveness and safety of medical products in unselected populations representative of the everyday clinical setting. However, clinical trials still represent the gold standard for securing regulatory approval of new drugs. Nonetheless, well-known disadvantages could preclude the adoption of trials. Moreover, recent medical interventions are often characterized by high costs that are sustained by healthcare systems. Therefore real-world evidence may also influence new policies about healthcare expenditures and drug reimbursements. The key challenges in analysing real-world data regard the significant amount of missing data with possible exposure misclassification, but most importantly outcome misclassification in the presence of confounding factors. Moreover, since real-world data can involve a wide range of research questions in healthcare, appropriate methodologies, including statistical models and machine learning algorithms, have to be investigated to combine clinical and administrative data. This thesis proposes a series of real-world case studies to address all these methodological aspects and empower the use of real-world data in the healthcare decision-making process. In the first project, the primary objective is the implementation of a data platform for designing, collecting, integrating and analysing health and administrative data extracted from hospitals' databases. The platform will serve as a tool to support clinical practice and measure the value of innovative cancer drugs. Furthermore, in the second project, a large real-world cohort of HIV patients is studied via machine learning algorithms to predict the risk of cardiovascular events possibly correlated with the use of antiretroviral therapies. In this case, the main focus is on the use of different analytical methodologies to discover new epidemiological insights about HIV therapies. In general, this thesis shows that to generate real-world evidence, new definitions of clinical outcomes must be adopted to monitor the real benefit brought by medical interventions. Moreover, it debates the reliability of artificial intelligence tools for outcome prediction and their actual advantages in clinical practice. Finally, the thesis addresses the quantification of health expenditures to investigate the cost-benefit relationship associated with new health technologies.

Keywords: real-world data, real-world evidence, precision medicine, Non Small Cell Lung Cancer, Ovarian Cancer, Human Immunodeficiency Virus, survival analysis, cost-effectiveness analysis, machine learning, deep neural networks.

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