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EDITORIAL

The importance of the pharmacoeconomic analyses in drug negotiation. A Farewell Editorial

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Decision analysis is considered an essential tool that every responsible decision-maker should use to make rational, informed choices that are ideally optimal according to predefined and possibly shared criteria. In the management of public healthcare, decision analysis tends to propose more cost-effective choices for the benefit of the community, operating in a specific context of limited healthcare resources that do not allow satisfying all patient needs. The use of decision analysis in the public healthcare context inevitably refers to the bioethical value of health equity: the most cost-effective choice aims to satisfy the maximum number of patients with the scarce available resources, adopting a utilitarian interpretation of the equity criterion.

Unfortunately, within the management of the public healthcare system, the use of decision analysis has faced challenges due to cultural, social, and organizational reasons. In Italy, the management of the National Healthcare System (SSN) and regions is structured into separate compartments (silos): pharmaceutical spending is managed separately from hospital and community care. Even today, managers of the pharmaceutical service must pursue the primary objective of keeping pharmaceutical spending within a predetermined maximum limit. During the early years of AIFA, this criterion hindered or at least discouraged the use of traditional pharmacoeconomic analyses in preparing Price and Reimbursement (P&R) dossiers for new drugs to be negotiated. The fundamental economic analysis required by AIFA was a simulated estimation of the budget impact of pharmaceutical spending in the first three years after the introduction of a new drug to the market. However, pharmacoeconomists from pharmaceutical companies progressively started submitting dossiers with BIA prepared according to international standards, which require evaluating the overall impact of the new drug not only on pharmaceutical spending but also on all types of healthcare costs (e.g., hospital costs, home care, etc.). Only in recent years, AIFA has explicitly valued cost-effectiveness analyses as a tool to support its decisions on drug prices and reimbursement.

The recent AIFA Monitoring Report 2022, prepared by the Economic Evaluations Office (Table I), highlights that in recent years, there has been a progressive increase in the percentage of P&R dossiers including pharmacoeconomic analyses. In 2022, one hundred six dossiers, equivalent to 62% of the dossiers submitted to AIFA, were accompanied by a pharmacoeconomic study: 105/106 (99%) included Budget Impact Analysis (BIA), 73/106 (69%) also featured a Cost-Effectiveness (CEA) or Cost-Utility (CUA) analysis, and only 1/106 (1%) exclusively included a CEA.

In summary, AIFA still considers BIA fundamental for negotiating the P&R of a drug intended for the Italian market. However, the percentage of dossiers including CEA studies is now significant and growing, particularly important for the negotiation of new drugs, especially orphan drugs. It should be emphasized that in the last two years, all dossiers for orphan drugs included a BIA and 86% also included a CEA. A similar trend, with slightly lower percentages, was observed for P&R dossiers of new chemical entities (Table 1).

Analyzing the BIA and CEA studies presented to support the negotiation of recent innovative drugs, especially some orphan drugs, reveals that these analyses can justify very high reimbursement prices that these drugs have obtained. For instance, the purchase cost of a CAR-T therapy has reached several hundred thousand Euros in Italy. When successful, a single-dose CAR-T treatment for a young patient allows for a normal life expectancy and accumulates healthcare savings for each avoided year of illness.

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	2018	2019	2020	2021	2022
Extensions of indication	21,00%	32,10%	30,60%	34,50%	44,10%
New Chemical Entities	61,30%	43,20%	87,20%	48,80%	94,60%
Orphan drugs	66,70%	54,50%	90,00%	100,00%	100,00%
Each type of negotiation	34,90%	37,10%	49,40%	44,60%	61,90%

Table 1. *Percentage of P&R Dossiers with Pharmacoeconomic Analysis and/or Budget Impact on the Total Number of Dossiers Submitted to AIFA in the Period 2018-2022, by type of negotiation*

This editorial is my fond farewell as "Editor in Chief" addressed to all the loyal readers of the journal Farmeconomia. Health Economics and Therapeutic Pathways, my colleagues on the Editorial Board, the publisher SEEd, and all the valuable contributors who made possible its birth 22 years ago and its continued development until today.

I am particularly delighted and honored to pass the baton to the distinguished Professor Francesco Saverio Mennini, whom I sincerely thank for accepting the responsibility of Editor in Chief. His leadership ensures not only the journal's robust continuity but also a perspective for further development as a means of communication for pharmacoeconomic research and the dissemination of accurate and updated health-economic knowledge. Professor Mennini is a renowned expert in health economics both in Italy and abroad and he holds significant scientific roles in the sector.

Pharmacoeconomics is an interdisciplinary scientific discipline that knowledge from pharmacology, medicine, health economics, social sciences, and bioethics. It primarily emerged from the need to justify the high cost of new drug therapies introduced into a healthcare market with limited resources and to support institutional decision-makers in making choices that ensure optimal health outcomes based on explicit and shared cost-effectiveness and equitable access to care criteria

In Italy, during the early 1990s, there were only a few experts in health economics, mostly from macro- and microeconomics disciplines, occasionally exploring themes related to the pharmaceutical market. At that time, clinicians and clinical pharmacologists were mainly interested in highlighting the benefits and risks of new drugs but were not inclined to consider their health-economic value or, more importantly, to base their therapeutic choices on cost or cost-benefit analyses. Additionally, drug prices and reimbursement conditions, covered by the public healthcare system and private entities, were negotiated at a political-administrative level, not always transparent, rational, or predefined.

Thirty years ago, as a clinical pharmacologist, I stumbled upon the field of pharmacoeconomics when an important international pharmaceutical company was about to introduce a new antibiotic into the Italian market, more effective against severe infections caused by resistant bacteria but also more expensive than existing options. Intrigued by the pharmaceutical company's proposal, I consulted some health economists and prepared a scientific communication project based on the cost and cost-effectiveness analysis of antibiotics. The project was accepted by the company and entrusted to a specialized publisher.

My economist friends provided me with the basic knowledge of microeconomics necessary to understand the techniques of pharmacoeconomic analyses. On my own, I studied the available international scientific literature and progressively learned decision analysis techniques applicable to studies in this field: decision trees, Markov chains, Markov chain Monte Carlo, Discrete Event Simulation, Bayesian statistics, Sensitivity Analysis, etc. My scientific career gradually shifted from clinical pharmacokinetics to pharmacoeconomics of antibiotics and, subsequently, to cost-benefit analyses of various types and classes of drugs. Thus, I became a pharmacoeconomist while always retaining my original training as a clinical pharmacologist. I transitioned from pharmacokinetic modeling to pharmacoeconomic modeling and had the opportunity to perform CEA, CUA and BIA of many drugs.

Gradually, I became aware of the complexity of the relationships between the efficacy-tolerability profiles of a pharmacological therapy and its economic and social implications on public and private healthcare systems. Moreover, I had to reflect on the substantial difference between the goals of scientific research (objectively establishing the efficacy, tolerability, and therapeutic position of a new drug) and the goals of decision research (supporting decision-makers in choosing among alternatives based on shared and transparent justice criteria, compatible with the economic and organizational conditions of a specific healthcare system).

After several years of work in this field, in 2000, I had the opportunity to found the journal Farmeconomia. Health Economics and Therapeutic Pathways, which I now entrust to Professor Mennini to whom I wish good work.