

# IMPACT OF THE ECONOMIC CRISIS IN THE APPROVAL OF NEW ONCOLOGICAL DRUGS – THE PORTUGUESE PARADIGM

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## Abstract –

**Background:** The European crisis lead to funding restrains in healthcare, already under pressure due to the ageing of the population and the increase of demands from innovation. Portugal is the paradigm of the European crisis since has both an economic and demographic crisis.

The researchers aimed to evaluate the impact of economic restrains in Portugal for access and reimbursement of new oncological drugs.

**Methodology:** A qualitative approach was used, supported by 27 formal, tape recorded, semi-structured interviews to representatives of the different healthcare stakeholders and policymakers. The content analysis with semantic associations through co-occurrence analysis were done with the support of Tropes® software.

**Results:** The results of the content analysis showed that economic restrains are leading to a policy of cost control with lower prices and reduced access to innovation; an excessive delay in the approval of new drugs; lack of transparency; serious limitations and inequity between hospitals. Contractual boundaries to national prescription was established and agreements with pharmaceuticals were made. Changes in the reimbursement process are being implemented with an increase of risk sharing mechanisms and implementation of a new system of health technological evaluation (SINATS). Treatment protocols are also being revised and public hospitals are trying to increase the number of clinical trials but there is still much bureaucracy. In this qualitative approach, the following factors with impact on survival were identified: Innovation and technological development, government funding, the price of drugs and type of oncological diseases.

**Conclusions:** The economic crisis is leading to a very serious problem of inequity. However, it is also an opportunity for a structural reform. In Portugal, an attempt of reform is being made with the implementation of SINATS since it is important to distinguish molecules that effectively bring added value. In order to consider the strategic vision in which the patient is the center of all efforts, the process of reimbursement approval for new medicines should be faster, more transparent and interdisciplinary. Moreover, the decisions must be done triangulating evidence based medicine, economics, health policy and ethical and legal issues. For National Health Service sustainability, efficiency and efficacy, clinical and economic reassessments must be done after market introduction of new medicines, with subsequent renegotiations.

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**Keywords:** Innovation; Economic Crisis, Healthcare.

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## I. INTRODUCTION

The healthcare cost of cancer care to European Union healthcare systems in 2009 was €51.0 billion, and accounted for 4% of total healthcare spending (1).

The regulatory agencies (such as the EMEA and FDA), governments, non- governmental organizations and patient associations have identified the most efficient route to market for health products. Formal network of health professionals help in providing feedback and data about health innovation (2) such as new drugs in oncology. Cancer is a priority for health systems around the world because of three main factors: a) the higher incidence, growing and aging populations; b) the impact of patients and caregivers productivity loss; c) and due to rising treatment costs (3).

A management model integrating budget restrictions, ethics, national and international law, society, pharmaceutical industry, policymakers, clinicians and patients is lacking. We need a new balance of values

and new ways of thinking and acting that must transcend national and institutional boundaries (4, 5).

Therefore, the Portuguese paradigm was chosen for two reasons: it has a national health service without charges for patient in oncology and it has a well-known European economic crisis with an ageing population. Our aim was to stablish the impact of crisis in the approval process of reimbursement of new drugs for oncology in Portugal accordingly with the different stakeholders and, based on that, to stablish strategic decisions to minimize the problems.

## II. METHODOLOGY

In 29 semi-structured interviews, 27 were tape recorded and transcribed to word between November 2013 and August 2014 (table 1). The ones made in Portuguese were translated to English. The interviews had an average duration of 35 minutes of recorded text (minimum 15 minutes and maximum 85 minutes).

**Table 1** – Interviews to the different stakeholders in the process of decision for new drugs in oncology

27 interviews (media – 35m; max . 85; min.15)	Duration
INFARMED, oncologist	35 minutes
WHO; sociologist	22 minutes
political decision maker, oncologist; ethics committee	30 minutes
Pharmaceutical director	20 minutes
Pharmaceutical director	20 minutes
Pharmaceutical director	33 minutes
Political decisor, economist	21 minutes
Head of oncology service; political decisor	18 minutes
INFARMED and hospitalar decisor	85 minutes
Oncologist	16 minutes
Oncologist	23 minutes
Head of oncology service; pharmacy commission	25 minutes
Pharmaceutical decisor	15 minutes
Patients association	25 minutes
Head of oncology service (islands)	16 minutes
Head of oncology service (central hospital)	26 minutes
Pharmaceutical maker	50 minutes
Pharmaceutical maker	25 minutes
APIFARMA; political consultant	42 minutes
Pharmacy commission; College of oncologists member	16 minutes
political advisor; former political decision maker	60 minutes
Oncologist; NOCs member	21 minutes
Oncologist; INFARMED	20 minutes
political decision maker	31 minutes
Former hospital director; Portuguese society of oncology	17 minutes
Former member of INFARMED and pharmaceutical consultant	42 minutes
Hospital decision maker; policymaker	23 minutes

A purposive sampling technique was applied to target the different stakeholders. The macro, meso and micro levels of health care are based on the WHO-definition (6). The macro level comprises the health system at a central level such as the regulatory agencies and government. The meso level comprises the health system at both the local and the organizational level, such as APIFARMA (Portuguese Association of Pharmaceutical Industry), INFARMED (National Authority of Drugs and other Health Products), hospitals and pharmaceuticals. The micro level includes the civil society and oncologists. Inclusion of the three groups was considered crucial to obtain individuals' perceptions, and information on interactions and on organizational levels of cancer care practices. Stakeholders were identified through informal and formal networking.

At least one representative of each group was interviewed and the researchers tried to choose interviewees with cumulative functions or that have already been in other functions besides the ones they have at the moment of the study. If invited stakeholders were unavailable or refused to be interviewed, another person, with a similar profile, were invited in a second wave of invitations. There were open (i.e. "What can be change?") and closed questions (i.e. like NICE?). The majority were open questions since we did not want that the different stakeholders to be limited.

The main questions were the following: 1) what are the main problems in the process of reimbursement of the new drugs in the context of European crisis? 2) what measures/actions should be or are been taken to reduce the costs of new medicines?

Semantic associations through co-occurrence analysis Tropes® software was used as support for content analysis and an external validation was made.

Table 2 - Dictionary of categories. The category system used was obtained with the support of the Software Tropes® Zoom V7, the English version (Cyberlex, 2003; Ferreira, 2008; Moltmann, 2004) and English V8.4

<sup>1</sup>NOCs – restricted clinical practice guidelines defined by Portuguese experts for the different diseases in oncology

<sup>2</sup>ARS - Regional Administration of Health

CATEGORIZATION	CODE	DEFINITION	EXAMPLE	FREQUENCY
ACTIONS AND INNOVATION	INNOVATION	Relation with innovation and actions	"For innovative therapy ... some hospitals do not adhere so quickly ..."	1116
BEHAVIORS AND FEELINGS	BEHAVIOR	Relation with feelings and behaviors from the different actors	"... doctors have always some uncertainty ..."	380
BUSINESS AND INDUSTRY	BUSINESS	Relation with pharmaceutical industry and health business	"... Measurement of added therapeutic value and its translation into economic and evaluation performed in a cost-effectiveness relation, which translates into QALYD ..."	1877
COMMUNICATION AND MEDIA	COMMUNICATION	Relation with health communication and scientific literature	"The NOCs are now a amount of paper that nobody uses ..."	248
COUNTRIES AND LOCATIONS	LOCATIONS	Relation with locations, countries and areas	"... Germany would also like to pay what Portugal pay for new drugs ..."	983
CRISIS AND CONFLICTS	CRISIS	Relation with economic crisis and several divergences	"The crisis is changing the mindset towards innovation in Europe ..."	198
EDUCATION AND WORK	WORK	Relation with work and academic degree	"... having a center that supports formation of oncologists as a way of monitoring the quality ..."	170
HEALTH, LIFE AND CASUALTIES	HEALTH	Relation with medicine, health, life, death and casualties	"... ensure that patients have access to the best treatment ..."	3287
LANGUAGE AND TEXTS	LANGUAGE	Relation with language, documents and papers	"... preceded by a discussion and a definition of crime ..."	193
NUMBERS, TIME AND DATES	NUMBERS	Relation with numbers, prices, rates, time and evaluation	"... it costs about 30 thousand euros a year ..."	910
PEOPLE AND ENVIRONMENT	PEOPLE	Relation with people and environment	"... Their committee had only people appointed by the political sector ... a mixed commission that had decision-making capacity ..."	710
POLITICS AND SOCIETY	POLITICS	Relation with politics, politic institutions and civil society	"... The ministry and the ARS continue as they were before ..."	1176
PROPERTIES AND CHARACTERISTICS	PROPERTIES	Relation with characteristics and/or properties of decision, actions or subjects	"... There is always a dose of uncertainty in making those decisions ..."	2372
SCIENCES AND TECHNOLOGY	SCIENCE	Relation with technology and science	"... Generate great know-how ..."	563
THINGS AND SUBSTANCES	THINGS	Reference to things and substances	"... 5 ampoules of treatment ..."	163

### III. RESULTS

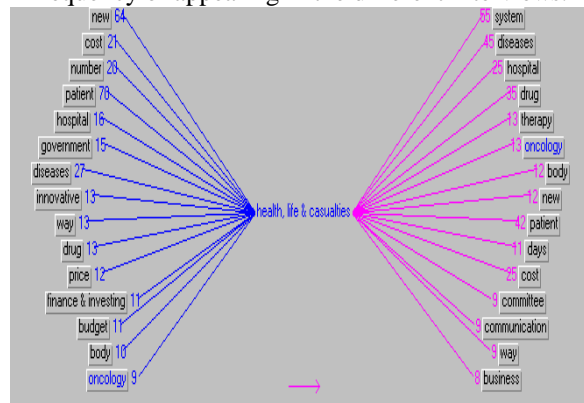
There are "major dynamics that contribute to rising health care costs" that are aging, scientific and technological advancement and the development of practices defensive medicine. The advancement of scientific and technological knowledge with the discovering of new therapies is extremely expensive and creates difficulties to the implementation of innovation..." (economist, political decision-maker). Portugal has adopted a "policy of controlling costs through prices" (APIFARMA decision maker) where "basically 49% of the adjustment was done around the new drugs..." This administrative policy is reflected in two levels "lower prices and reduced access" (APIFARMA decision maker). The "exponential increase in the cost of drugs is a problem identified worldwide" although Portugal is the paradigm of a European crisis with lack of money "(INFARMED decision maker). However, in the last two years, Portugal was "among the three lower European countries in terms of per capita public expenditure per patient "(APIFARMA decision maker). The "pharmaceutical industry through APIFARMA" signed an agreement with the government, the ministry of health in order to control spending. They established a ceiling and all the value that exceeds a certain value is returned by pharmaceutical companies directly to hospitals in credit notes" (pharmaceutical decision maker).

It is important to "distinguish what are those molecules that effectively bring added value, therapeutic benefit and quality of life for patients from those molecules that are mere novelties" (pharmaceutical decision maker).

There is an "excessive delay in the approval of new drugs, serious limitations and a lack of equity at a nationally level." (pharmaceutical decision maker). The lack of equity is consensual in our study. It is still referred the need for greater transparency in the decision-making process." (sociologist; WHO). The funding system in Portugal is a system of the most ancient in terms of substantiating a drug-economic evaluation. (APIFARMA decision maker). It is important demonstrate the added therapeutic value of the new oncological drugs and risk sharing mechanisms and to investigate the safety and efficacy of drugs after its approval (APIFARMA decision maker). The "risk-sharing models" has been advocated either by policy makers or by decision makers in the pharmaceutical industry and is already being implemented in Portugal with SINATS (hospital decision maker) will be an option and a way to cut costs at a public level. In fact, considering the rising costs in oncology, the government tries to block the access to innovation" (pharmaceutical decision maker) and "the pressures are such that the physicians often do not use the product ... "(pharmaceutical decision maker). Policy makers claim to have "limited resources" (political decision maker). In addition, hospitals have "a calculated budget in a certain way" and, with the advent of new drugs such as those for hepatitis C, "suddenly has to spend another 500 million or 500,000 or half a million without knowing how." (political decision maker; oncologist). Funding is one of the most complex issues and should follow the patient as several stakeholders mentioned something that is not currently done. Clinical trials are a way of getting decrease the economic impact of drugs and a way of getting to provide treatment to patients spending less and I think this is a way to make it more accessible innovation, a way that is correct because it also brings additional information" (head director of an oncology service). Portugal is a "country of reference for many countries in Europe" (pharmaceutical decision maker) and access difficulties to new drugs have to do with the phenomena of parallel exports of drugs to other countries" (APIFARMA decision maker). Patients' associations try to defend patients and citizens interests so, "they must have interconnection with the processes of health" and be more active in the decision processes (civil society member). Portugal is "not prepared to deal with the huge increase of innovation" and some measures are being implemented such as the creation of SINATS which is a system of health technological evaluation that are proposed to be implemented during 2015 and 2016 by INFARMED (Hospital decision maker and political decision maker). Innovation and technological development, costs, hospitals with patients and diseases as well as prices, particularly of new drugs, are the main factors that will influence the health and survival. Consequently the state of health of the citizens will influence not

only the system and cost, but also hospitals, the intake of drugs, treatments, communication and business (chart 2).

Chart 2 - Factors that influence health, life and casualty and its consequences with the respective frequency of appearing in the different interviews.



## DISCUSSION AND CONCLUSION

In our study, it is possible to identify two main problems of oncology in Portugal which can be also present in other European countries namely, the cost of new oncological drugs and the scientific development with a huge increase of innovation (7, 8).

As confirmed in our study, the three major dynamics contributing to the rising costs in health are the increasing demand of an ever-expanding and ageing population (9, 10, 11), technological breakthrough (12) and scientific innovation and the development of defensive medicine (13, 14).

The growing aging of the Portuguese population until 2050 put increasing pressure on public finances by increased expenditure on pensions, elderly care and also in health (15, 16).

Although equity of access to health care is a central objective of many health care systems, this is not always possible because socio-economic constraints (17, 18). In Portugal there is a very serious problem of inequality and economic constraints which are leading to changes in the process of reimbursement with the formation of the National Commission of Pharmacy, creation of high differentiation centers and the implementation of SINATS (19, 20). SINATS is an open model, with active participation of all stakeholders which aim the maximization of gains in health and quality of life, contribute to the sustainability of the National Health Service, ensuring the efficient use of public health resources, monitor the use and effectiveness of technologies, reducing waste and inefficiencies, promote and reward the development of relevant innovation (21).

This new model aims to assess other aspects of the value of technology in addition to the added value and economic value and will do the evaluation after market introduction and it is the most relevant change

in the process of reimbursement (22). The question is if this model will allow the alignment between approval and introduction of new drugs with simplification of the process. The fear of many clinicians and society is that the measures that seem beneficial "ad initium" may be too restrictive and prevent the prescription of drugs already approved and marketed in Europe for its clinical benefit (23).

Health decisions that allow the reduction of costs cannot be ethically responsible (24). It is important to implement corporate social responsibility (CSR) which "is not only doing the right but also make things better" (25). In health organizations there is evidence that, for the implementation of organizational changes, the agents have to overcome the resistance of the other members of the organization for the adoption of new practices (26). So, whereas the decision for the approval of new cancer therapies should be based on ethics, integrating civil society and consider the patient (the main "stakeholder") (27), it is important to implement changes that integrate CSR in Portuguese model (even if there is resistance on the need to change the organizational culture). However, without a structural reform in the National Health Service it will be difficult to maintain a high quality level in the area of oncology without compromising the economic sustainability. Moreover, a change in the system of evaluation for innovative drugs is important for all European countries in order to maintain sustainability.

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