Pharmacoeconomic Evaluation of Orphan Drugs in the United Kingdom, France, Italy, Turkey and Exploration of the Evaluation Process of Orphan Drugs in Algeria.

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Introduction

Access to medicines is a fundamental element of the right to health, it must be guaranteed, because it represents an ethical issue

To achieve this objective, States around the world have developed various tools and mechanisms to guarantee this right to their populations according to their capacities and their health systems.

Access to the treatment of rare diseases is no exception to the rule and raises even more challenges due to the difficulties of diagnosis and treatment.

Objectives

- Compare the different processes of pharmaco-economic evaluation of orphan drugs in four countries: France, Italy, the United Kingdom and Turkey
- Exploring the orphan drug evaluation process in Algeria.

Methodology

An extensive review of literature was carried out to collect information on the evaluation models of different orphan drugs in the four countries. The literature search was carried out on the official websites of the agencies and organizations responsible for the regulation of medicines in each country. The analysis was undertaken through several aspects including: the existence of specific legislation for the evaluation of orphan drugs, pricing, reimbursement, and application of health technology assessment (HTA).
Finally, a semi-structured interview was conducted at the National Agency for Pharmaceutical Products in order to explore the evaluation process of orphan drugs in Algeria.

Results

Country	Pricing	Reimbursement	HTA	Existence of national plan for orphan drug
France HAS HAUTE AUTORITÉ DE SANTÉ	• Same procedure for non-orphan drugs (according to ASMR) • Generaly ASMR I- III for orphan drugs, which means high price • Almost all innovative drugs enter the French market under a price-volume agreement/contract.	• Same procedure for non-orphan drugs (according to SMR) • More flexibility regarding the strength of evidence.	• Same procedure for non-orphan drugs. • Exemption from health economic evaluation applied to orphan drugs with a low budgetary impact (<30 million euros/year for a particular indication)	Yes
Italy AIFA	• Same procedure for non-orphan drugs • Flexibility regarding: pricing, regulation, clinical data requirements and level of clinical uncertainty. • High price could be accepted • The price is negotiated between the AIFA and the manufacturer, confidential discounts and rebates may also apply for orphan drugs.	• Same procedure for non-orphan drugs • Most orphan drugs are distributed through hospitals.	• Same procedure for non-orphan drugs.	Yes
United Kingdom NICE National Institute for Health and Care Excellence	• Same procedure for non-orphan drugs • Free price but must meet the benefit control criteria also applied to non-orphan drugs.	• Same procedure for non-orphan drugs • NICE bases its decision on cost-effectiveness, although other criteria, such as fairness, may also be considered.	• Same procedure for non-orphan drugs but NICE accepts greater clinical uncertainty. Acceptability threshold is high	Yes
Turkey T.C. SAĞLIK BAKANLIĞI TÜRKİYE İLAÇ VE TIBBİ CİHAZ KURUMU	• Same procedure for non-orphan drugs.	• Same procedure for non-orphan drugs. • All orphan drugs that succeed in entering the market are reimbursed, regardless of the granting of an MA	• Same procedure for non-orphan drugs • HTA studies are conducted in Turkey to assess the clinical efficacy of orphan drugs which are exempt from pharmaco-economic analysis.	No

In Algeria

Abbreviated procedures for documentary assessment during registration of these medicinal products were introduced on 27/12/2020. It is recommended to develop a procedure for pharmaco-economic assessment or specific incentive measures that will improve the access of Algerian patients to these treatments. It is also recommended to create a health technology assessment body to provide more data and help decision-making.

Discussion

Worldwide, the management of rare diseases represents a real public health challenge. Orphan drugs intended for the treatment of diseases should follow specific procedures different from those of other drugs for obtaining their market authorisation, fixing the price and reimbursement. It should be noted that rare diseases strongly affect the lives of patients and most often remain without therapeutic response and require rapid and appropriate care and therefore rapid access.

For this, and in order to guarantee fair access to treatment for patients, several countries around the world have implemented more specific measures and procedures that have demonstrated their effectiveness, guaranteeing early access to the market and using various modes of financing (performance contracts) following the example of Italy and France. The United Kingdom uses the Health Technology Assessment approach to better estimate the value of orphan drugs by applying relevant methods such as cost-effectiveness and budget impact. Turkey, on the other hand, having the closest health system to that of Algeria, has introduced more flexible measures to facilitate the reimbursement of this category of medicines in order to better meet the needs of the patients concerned

While in Algeria, the evaluation of orphan drugs and the setting of prices goes through the standard procedure, which delays the availability of these drugs on the market. However, with the creation of the Ministry of Pharmaceutical Industry and the restructuring of the National Agency for Pharmaceutical Products, and the strengthening of the regulatory framework governing the evaluation of drugs, and the integration of the concept of pharmaco-economic evaluation an improvement in the evaluation of drugs is expected. For orphan drugs, this is still insufficient, a broader reflection on regulatory assessment, prices and financing methods should be undertaken.

Conclusion

Different drug evaluation approaches and processes are applied around the world. Each country is constantly reviewing its evaluation system with a view to optimizing it in order to guarantee patient access to medicines, in particular orphan medicines.

Since access to drugs is a performance indicator for a health system, Algeria should draw inspiration from foreign models to put in place specific measures applicable to the registration process for orphan drugs, and diversify funding methods. . It is also important to strengthen the system through a more comprehensive drug evaluation process by integrating the Health Technology Assessment "HTA" approach.

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Abbreviations and definitions: **SMR:** The Actual Benefit is a criterion that informs the authorities in charge of admission to reimbursement of drugs on the clinical benefit of drugs. • **ASMR:** improvement in actual benefit is an assessment used by the Transparency Commission of the French National Authority for Health to gauge the benefit of a new drug in improving actual benefit in a therapeutic strategy. • **HTA:** Health Technology Assessment is a multidisciplinary field of activity that aims to assess drugs, devices, methods and modes of intervention used for the prevention, diagnosis, treatment of health problems, or for rehabilitation. • **HAS:** In France, the Haute Autorité de Santé is an independent public authority that contributes to the regulation of the health system through quality. • **NICE:** National Institute for Health and Care Excellence which provides health guidance, counseling and information services in UK • **AIFA:** Agenzia Italiana del Farmaco is the national medicines agency in Italy • **TITCK:** Türkiye İlaç ve Tıbbi Cihaz Kurumu is the National Medicines Agency in Turkey