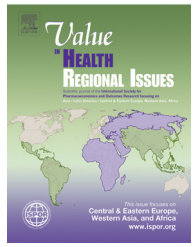
VALUE IN HEALTH REGIONAL ISSUES 4C (2014) 47–52

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/vhri



An Overview of the Orphan Medicines Market in Turkey

Güvenç Koçkaya, MD, MsC[1,], Albert I. Wertheimer, MBA, PhD[2], Pelin Kilic, MsC[3], Pelin Tanyeri, MD[4], İ. Mert Vural, MD[3], Akif Akbulat, MD[3], Guven Artiran, MD[3], Saim Kerman, MD[3]*

[1]*Health Economics and Policy Association, Ankara, Turkey;* [2]*Temple University, School of Pharmacy, Philadelphia, PA, USA;* [3]*Turkish Medicines and Medical Device Agency, Ankara, Turkey;* [4]*Sakarya University, Medical Faculty, Pharmacology Department, Sakarya, Turkey*

ABSTRACT

Background: The health policies of many countries and regions have already defined orphan drugs for rare diseases. Although there is no official definition of orphan drugs in Turkey, all orphan drugs are covered by reimbursement, regardless of their market authorization status. Thus, a pharmacoeconomic analysis does not have to be presented to the Social Security Institution (Sosyal Güvenlik Kurumu) for reimbursement decisions on orphan drugs. **Objective:** The aim of this study was to shed light on the use of orphan drugs to aid classifications of rare diseases and assessments of orphan drugs in Turkey. **Methods:** Data for sales of authorized orphan drugs and all other drugs were extracted from the IMS Turkey for 2008, 2009, and 2010. Nonauthorized orphan drug sales data were extracted from records of the Turkish Pharmacists' Association for the same years. Government prices were obtained from the Sosyal Güvenlik Kurumu. **Results:** The European Medicines Agency has classified more than 60 orphan drugs for rare diseases. Of these, 50 entered the Turkish market in recent years, half of which were authorized. The remaining drugs were imported through the early access procedure. Antineoplastic agents accounted for the largest percentage of orphan drugs, with 58% of the total market share. In 2010, there were 18 such agents in use, at a cost of €120 million. **Conclusions:** Although legislation is not yet in place for orphan drugs in Turkey, recognized pricing and reimbursement policies are in operation. This situation facilitates an analysis of orphan drug prices and reimbursement policies in Turkey.

Keywords: early access, orphan drugs, Turkey.

Introduction

It was reported in a recent review that 5400 new molecule entities were in the clinical research process in 2013 [1]. Research on rare diseases is one of the fastest growing areas in medicine in recent years. A total of 1795 projects about rare diseases were reported as being in the development process [1]. This research may increase in the coming years. A substantial part of the research on orphan drugs for rare diseases is led by small pharmaceutical and biotechnology companies because such drugs are potentially easier to get onto the market than are drugs are intended to treat widespread diseases and conditions. This is due to several reasons—for example, the well-known etiology, hence the clinical interest and expertise to treat common diseases, and the fact that common drugs are launched at much lower prices than are orphan drugs. Orphan drugs are intended to be used in a country or region in the same way as regular medications (e.g., antihypertension medications). Although there is high demand for orphan medications for specific diseases, it is much more difficult to design clinical research for orphan drugs than for other drugs because of low numbers of the target patient population.

Rare diseases are defined in the United States as diseases or conditions affecting fewer than 200,000 individuals [2,3]. The European Union (EU) defines rare diseases as life-threatening or chronically debilitating conditions that affect no more than 5 in 10,000 people [4–6]. Definitions of orphan drugs are used for classifying medicinal products intended to treat rare diseases.

The Orphan Drug Act of 1983 defined a pathway through new product development: with data supporting the treatment of a rare disease or condition, a medicine can receive orphan designation status in the United States [7–9]. The EU implemented regional policies in 2000 with the aim of improving research and development in the field of orphan drugs [4,5,10]. According to one study, an application for an orphan status designation from the US Food and Drug Administration (FDA) is perhaps the simplest and most straightforward request that can be made to the FDA [8].

Although the cost of launching orphan drugs for rare diseases is similar to that of launching drugs for common diseases, the low patient numbers increase the price of orphan drugs. Because of the inverse proportion between patient numbers and prices, no alternative health technology exists for many orphan drugs [5]. This is, of course, one bottleneck in the course of orphan drug development. To manage this situation, many countries and regions around the world offer economic incentives to make orphan drug development attractive, especially to small and medium companies [11–13].

Conflict of interest: The authors have indicated that they have no conflicts of interest with regard to the content of this article.

* *Address correspondence to*: Güvenç Koçkaya, Saglık Ekonomisi ve Politikası Derneği, Ufuk Üniversitesi Cad. No:3 The Paragon K:23 Söğütözü/Ankar, Turkey.

E-mail: guvenc.kockaya@sepd.org.tr

2212-1099$36.00 – see front matter

http://dx.doi.org/10.1016/j.vhri.2014.06.009

The cost of most orphan drugs is higher than that of conventional drugs. In addition, the lack of clinical data on orphan drugs restricts pharmacoeconomic assessments. Both make reimbursement difficult. The first-line approach to aid reimbursement decision making on orphan drugs is to conduct additional assessments after the enactment of orphan drug legislation [14].

One hundred twelve different active substances are listed as orphan drugs by Orphanet [15]. The FDA and the European Medicines Agency (EMA) have already classified a number of rare diseases and orphan drugs, with the EMA authorizing 60 different orphan drugs to date [16,17]. Although there is no official definition of orphan drugs in Turkey, a working group has been established and is used in establishing laws and definitions related to rare diseases.

The Turkish Medicines and Medical Device Agency—Türkiye İlaç ve Tıbbi Cihaz Kurumu (TITCK)—is responsible for the pricing of pharmaceuticals. All drugs in Turkey are subject to a reference pricing policy, with France, Greece, Italy, Portugal, and Spain used as the reference countries since 2006. The Social Security Institution—Sosyal Güvenlik Kurumu (SGK)—is responsible for reimbursement. Two scientific commissions that are responsible for setting opinion for reimbursement operate in the evaluation of such dossiers submitted for the pricing of medicinal products. The Medical and Economic Evaluation Commission assesses all orphan drug applications and conveys its findings to the Reimbursement Commission, which finalizes the decision made by the Medical and Economic Evaluation Commission. The Medical and Economic Evaluation Commission and the Reimbursement Commission consist of the TITCK, SGK, and the Ministry of Finance. All reimbursement applications submitted to the SGK for conventional drugs must include a pharmacoeconomic analysis [18] (Fig. 1). Orphan drugs are exempted from this requirement. In accordance with the Notification on the Pricing of Medicines, the TITCK sets the price of these drugs using the reference prices, as documented by the official records of the country of manufacture or export (this is considered to be the original price). The SGK reviews orphan drug prices annually and revises them on the basis of their sales volume. All orphan drugs entering the market are reimbursed without any co-payment. Because the TITCK does not have to present a pharmacoeconomic analysis to the SGK for reimbursement decisions of orphan drugs, such drugs may enter the market faster than conventional drugs [19].

However, most of the orphan drugs accessed in the country, which are not licensed by the TITCK, may be in the hands of the responsible physician and the patient in need with the early access procedure defined by the TITCK. This procedure allows physicians to prescribe off-label medications or unlicensed drugs. The TITCK evaluates off-label and unlicensed medication use for each patient through off-label application procedures. A treating

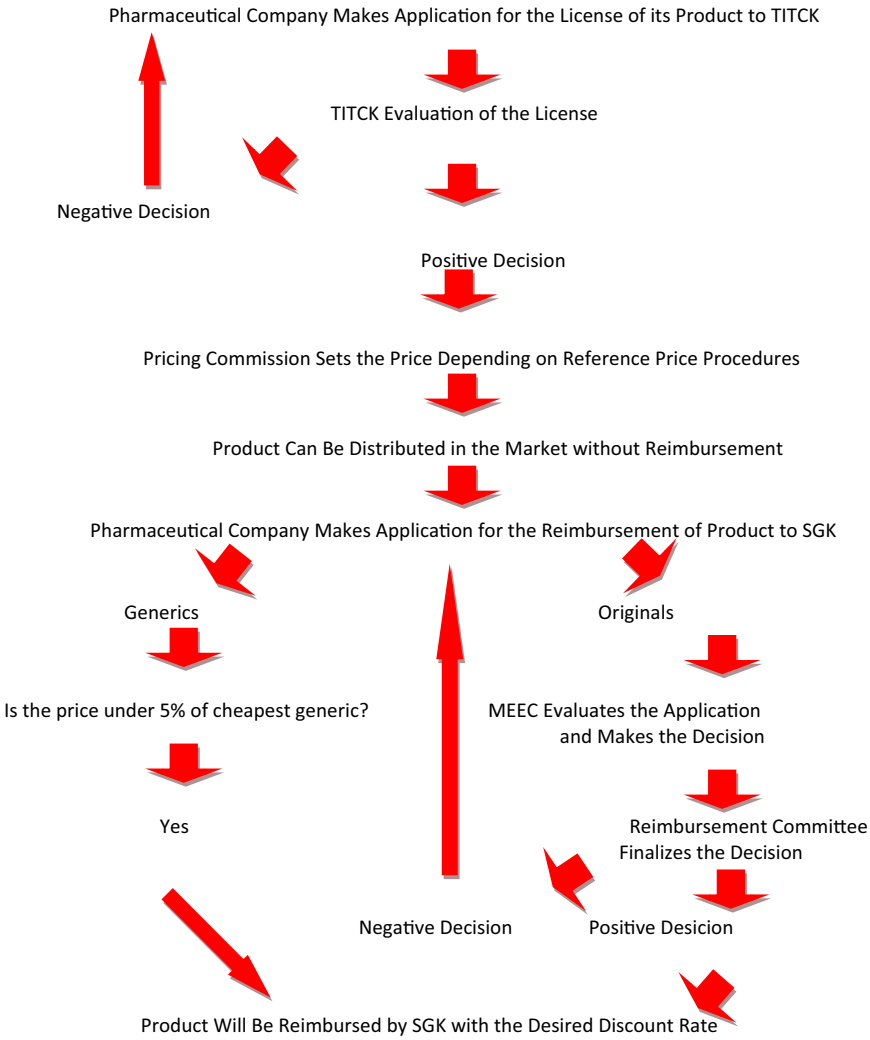


Fig. 1 – Overview of licensed product market access in Turkey [17]. MEEC, Medical and Economic Evaluation Commission; SGK, Sosyal Güvenlik Kurumu; TITCK, Türkiye İlaç ve Tıbbi Cihaz Kurumu.