SERVIZIO SANITARIO REGIONALE
EMILIA-ROMAGNA

RegioneEmilia-Romagna

# DELIVERY OF DRUGS FOR RARE DISEASES IN EMILIA-ROMAGNA REGION

**M. Volta, E. Rozzi, M.T. Montella**

**Servizio Assistenza Ospedaliera, Direzione Generale Cura della Persona, Salute e Welfare, Regione Emilia-Romagna**

## OBJECTIVES

Italian law provides for the identification of the clinical tests and drugs to be granted free of charge to patients. Rare diseases (RD) require several drugs which are normally paid; **Emilia-Romagna region has defined paths to ensure the free and appropriate supply of these drugs.**

## METHODS

Emilia-Romagna Region has developed **two procedures of authorization to grant the free supply of drugs**: the first one applies to all carriers of specific diseases, and it passes through a regional act; the second one applies to individual patients and involves an evaluation by a Rare Diseases Regional Committee (GTMR).Both the procedures are based on scientific literature and clinical practice.

## RESULTS

Since the introduction of the treatment plan in the Information System (June 2011) to 31/12/2015, **13,290** individual treatment plans (PTPs) have been reported, referring to **4,336** patients and comprising a total of 36,323 prescriptions. Emilia-Romagna regional acts have approved treatment protocols for neurological rare diseases, dermatological rare diseases, ophthalmic rare diseases, metabolic rare diseases, as well as interstitial cystitis.

The regional acts approving these protocols report lists of medicines which are essential for patients, thus proposing their free supply, and lists of medicines that were not effective or did not show sufficient evidence of safety, and therefore to be excluded by the free-of-charge distribution system.

**The Rare Diseases Regional Committee (GTMR) evaluated 40% of PTPs** (5,310/13,290).
**Committee's evaluation was positive in 83%** of the cases. (Figure 1)
63% of applications are referred to drugs, 30% to supplements, and 7% to medical devices. (Figure 2)
Data analysis on pharmaceutical costs shows that the total cost on drugs for rare diseases amounts to **more than 141 million €** in the period 2005-2015. Approximately **19% of this budget (26 million €) is referred to drugs approved by GTMR**

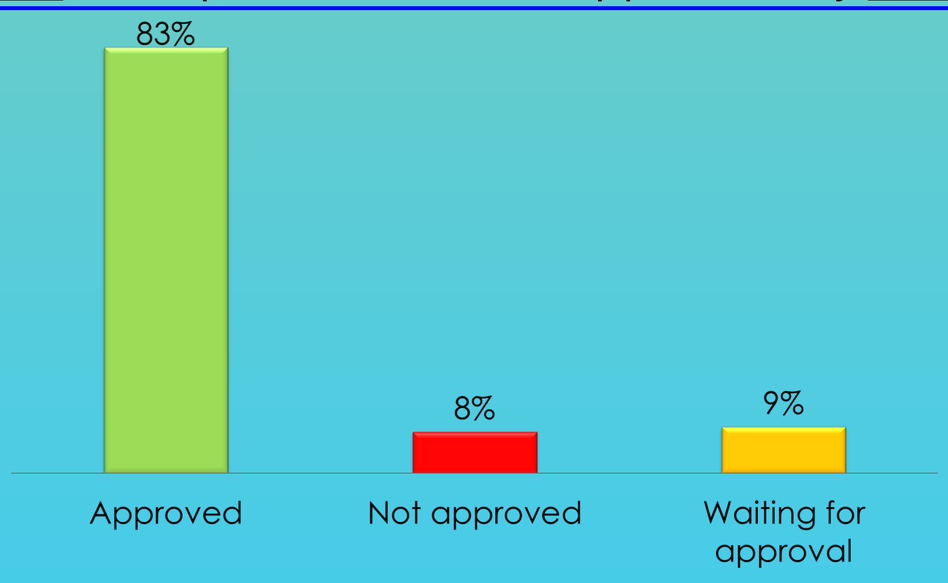


**Figure 1. Evaluation results**

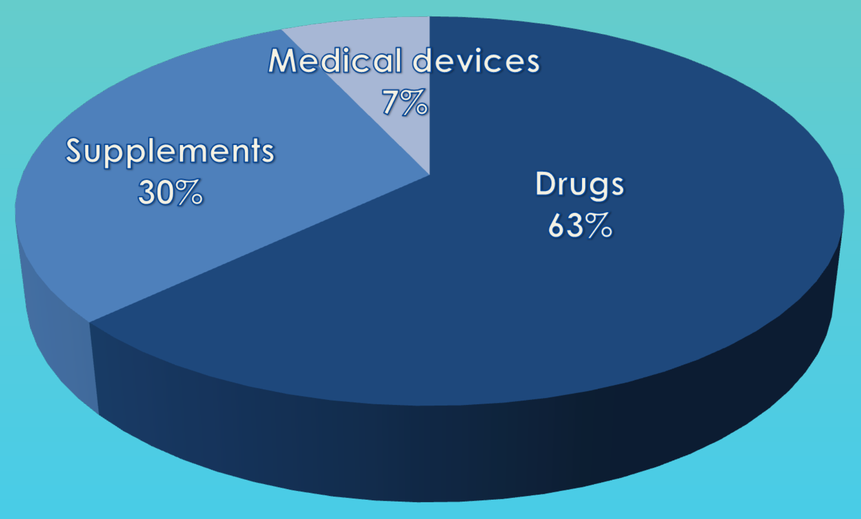


**Figure 2. Applications to GTMR**

## CONCLUSIONS

**The development of special paths for patients with rare diseases allows the free supply of drugs, supplements and medical devices** that would be otherwise charged to the patients themselves. The decision to approve regional acts that define protocols identified by clinicians experts in rare diseases, and to rely on GTMR, ensures **proper treatments** and **consistent modes of action**.

The high number of applications clearly shows the **need for these patients to receive free-of-charge drugs**.

The regional costs related to drugs for rare diseases is significant and the **computerization of treatment plans** allows for **constant monitoring to ensure an effective health system management**.

26-28 May 2016 Edinburgh
European Conference on Rare Diseases & Orphan Products

**For further information please visit the site**
**http://salute.regione.emilia-romagna.it/assistenza-ospedaliera/malattie-rare**
**or contact us at malattierare@regione.emilia-romagna.it**