

# A web-based system for the prescription, delivery and monitoring of treatments for rare diseases patients: the experience of the Veneto Region Coordinating Centre for Rare Diseases

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## BACKGROUND & OBJECTIVE

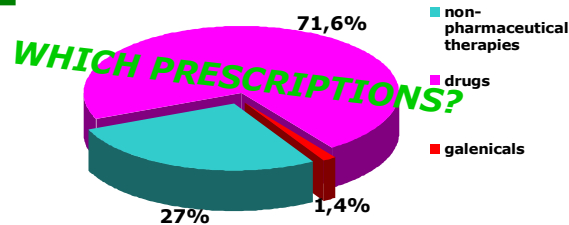
One of the problems rare diseases patients face is the limitedness of treatment options. Even when treatments exist, patients experience difficulties in accessing therapies because of scarce information on prescription modalities, the necessity to follow complex administrative procedure to obtain therapies, especially if free of charge, and the involvement of clinicians from different specialties in the care process, each one managing an aspect of the global treatment. The process from prescription to treatment access involves many actors: clinicians of specialized Centres, general practitioners (GPs), pharmacists working in hospitals, local health districts and private pharmacies. **Objective:** to implement new functions in the rare diseases system to prescribe drugs, dietetics and other medical products connecting the prescription, delivery and administration processes in particular of orphan drugs, galenicals, in order to support strategic health planning in the wide area for rare diseases.

## HOW MANY PRESCRIPTIONS IN THE VENETO REGISTER?

In 2009 more than **3,150** patients had a therapeutic plan prescribed through the IS.

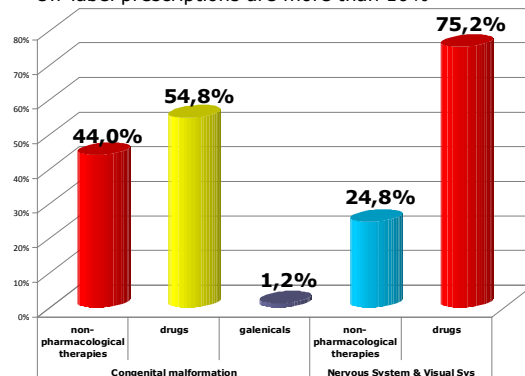
## MATERIALS & METHODS

In the Veneto Region a web-based system accessed by all these actors has been designed to manage every aspect of the treatment pathway of RD patients. It is part of existing Regional Register of Rare Diseases, a monitoring system completely computerized supporting the activities of the RD care network. Data regarding treatments' prescriptions are recorded in the rare diseases system by clinicians of Reference Centres in the electronic therapeutic programme. Therapeutic protocols elaborated by the reference centres are accessible and shared through the informative system. Prescriptions are visible by GP and pharmacists, who directly provide treatments to patients according to their place of residence (figure below). The Anatomical Therapeutic Chemical classification System (ATC) is used to register drugs' prescriptions.



RD patients do not need only drugs but also medical devices, dietetics, galenicals, that is personalized therapies.

Off-label prescriptions are more than 10%



## RESULTS

Specific sections have been developed for the prescription of dietetic and galenic products and other non-pharmaceutical therapies, i.e. rehabilitation treatments. The system can be used also to report adverse events and prescribe exams or lab-tests necessary for therapy monitoring. A distinct sub-section has been specifically designed for the prescription of orphan drugs and their post-marketing surveillance.

Figure. The process from prescription to treatment access by monitoring regional system for rare diseases

1. RD patients refer to Reference Centres



2. Clinician records prescription in the web-based system. Therapeutic protocols elaborated by the reference centres are accessible and shared through the informative system.



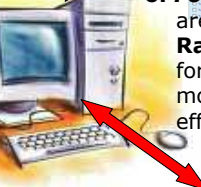
6. Follow up schemes are implemented in the Rare Diseases Register for **ORPHAN DRUGS** to monitor treatments' effectiveness and safety



3. Hospital Pharmacist prepares galenicals



4. Hospital pharmacist provides treatments to patient



5. Drugs administration (i.e. enzyme replacement therapy, ERT) in a hospital near patients domicile or in an outpatient clinic of local health districts

## RARE DISEASES GROUPS WITH THE HIGHEST NUMBER OF PRESCRIPTIONS

WITH THE HIGHEST NUMBER OF PRESCRIPTIONS

**ORPHAN DRUGS: CRUCIAL ISSUES** Drugs development for these conditions has been limited by a lack of understanding of the underlying mechanisms of diseases and the relative unavailability of subjects for clinical trials, as well as the prohibitive cost of investing in a novel pharmaceutical agent with poor market potential. The introduction of Orphan Drug legislations has provided important incentives for the development of orphan drugs (EU Regulation No. 141/2000). Orphan drugs are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions that affect no more than **5 in 10,000** people in the EU. The potential effect on health care systems due to high cost treatments and increasing number of eligible patients needs to be evaluated in terms of real impact on the quality life of patients, of really efficacy in clinical practice and of global sustainability.

## A MONITORING FUNCTION IN THE SYSTEM

## CONCLUSIONS

The IS, while connecting all the actors involved in the treatment prescription process, simplifies the patients' care pathways in accessing therapies. At the same time it generates important clinical information on treatments' effectiveness and safety and reimbursement costs, fulfilling also a monitoring function. The implementation of post-marketing monitoring systems is necessary to support the sustainability of the health systems as well as drugs' accessibility in the medium and long-term period, allowing also the modification of the drugs' market prices, according to their real utilization both at a national and at a EU level.

