

Galen Help

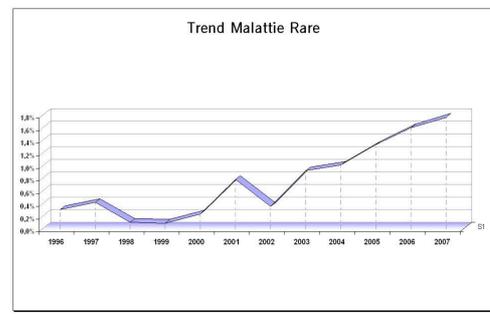
Help by the Pharmacist for Rare Diseases

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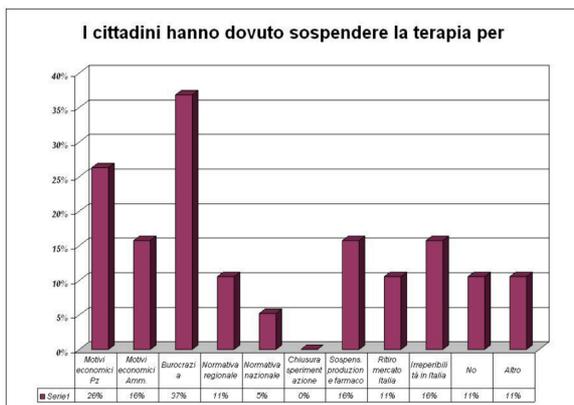
¹The Professional Pharmaceutical Union for Orphan Drugs (U.P.F.A.R.M), ²Italian Federation Rare Diseases (UNIAMO FIMR onlus)

The "Galen Help. Help by the Pharmacist for Rare Diseases" project is collaboration between the Italian Federation Rare Diseases (UNIAMO FIMR onlus) and the Professional Pharmaceutical Union for Orphan Drugs (U.P.F.A.R.M). Its purpose is to offer a concrete help to those patients, and to their families, affected by rare diseases and in search of specific pharmacological treatments to address their unique condition. Unfortunately, the availability of such treatments is inadequate to satisfy the demand because they are no longer mass-produced for economic reasons. This shortage occurs especially in pediatrics where such drugs require non-standard dosages. Therefore, with the availability of the raw components, a vast majority of such drugs can be prepared in the Galenic pharmaceutical laboratories on an individual basis: A unique prescription tailored to a specific patient. Currently, two-hundred pharmacies distributed throughout Italy take part in the "Galen Help. Help by the Pharmacist for Rare Diseases" project, making possible for patients to obtain drugs such as Nadolol, Mexiletine, etc., which are essential to their health and cannot be substituted by other drugs. The model developed by the "Galen Help" project can be adopted in Europe. One of the goals of this project is to involve the European Community, through the creation of a dedicated Committee to coordinate the initiative, in assigning to the licensed pharmacists of each European country the preparation of orphan drugs. The model can be replicated in those countries which present a situation similar to the Italian one, namely a lack of distribution of such drugs on a large scale. Thus, facilitating patients and allowing substantial savings for government health expenditures.

The Italian Annual Reports assessing the relationship between citizens and medical services offered reveal an increasing trend—the number of occurrences has doubled in the last ten years—and highlights that the most difficulties pertain to accessing: information, drugs, timely diagnosis, adequate care and innovative treatments.



TREND RARE DISEASES
Source Cittadinanzattiva - 2007 PIT Health Report



REASONS PATIENTS HAVE BEEN FORCED TO INTERRUPT THERAPY
Source Cittadinanzattiva - 2008 Study

The "Galen Help. Help by the Pharmacist for Rare Diseases" project originates from the great need of rare disease patients and their families, as a new opportunity to address the inadequacy of orphan drugs availability and distribution, as well as its deficiencies regarding dosages and type of drugs. In fact, patient associations receive reports regarding the interruption of pharmacological treatments due to drug unavailability in Italy (16%) caused by either discontinued production (16%) or withdrawal from the Italian marketplace (11%). The gap from the need to the possible solution has been brief: With the availability of the raw components, a vast majority of such drugs can be prepared in the Galenic pharmaceutical laboratories on an individual basis: A unique prescription tailored to a specific patient. This method allows, through the rationalization of the network between Pharmacies and Associations for Rare Diseases, the manufacturing of specific drugs for only one or even two patients within the same country, something that could not be possibly fulfilled by the Pharmaceutical Industry.

Thanks to the agreement between UNIAMO, the Italian Federation Rare Diseases onlus and U.P.F.A.R.M, the Professional Pharmaceutical Union for Orphan Drugs, a national network of accredited privately owned pharmacies adhering to the "Galen Help. Help by the Pharmacist for Rare Diseases" project has been established. Such network executes the goals of the project. The affiliation of such pharmacies to the "Galen Help" network is open and voluntary, as long as the technical requirements for the accreditation are met based on the protocol defined by U.P.F.A.R.M. A database has been created in order to indicate to the needy patient the closest pharmacy within the Galenic help network. This database is constantly updated and it can be accessed from the websites of UNIAMO (www.uniamo.org) and U.P.F.A.R.M's (www.upfarm.it). In addition, the "Galen Help" project will promote drug awareness and training through seminars and industry events, with the involvement of universities and regulatory bodies, as well as the participation of national and European institutions. This project does not have the presumption of resolving the problem of orphan drugs by itself. Its goal is to provide help to patients afflicted by rare diseases, making available to them those drugs that can be prepared in a Galenic pharmaceutical laboratory and which do not require highly sophisticated production techniques, thus ensuring the availability in procuring such drugs, safety in their making and a fair price. The unique production of those specific drugs by Galenic pharmaceutical laboratories, which do not interest the commercial industry, could bring a reduction of costs sustained by the national health services and allow a recovery of funds to be used in the research of new treatments for rare diseases. The intended goal is to expand the "Galen Help" project on a European level and to create a coordinating Committee with the function of indicating the manufacturable drugs within the Galenic pharmaceutical laboratories available within the different European countries. The legitimacy of this project, considering also the benefit from the reduction of expenses by social health services in this particular downturn of the economy, would advocate the pharmacy as a favorable substitute, for its specific competence, in handling the pharmaceutical needs of patients affected by rare diseases, which would have at their disposal not only a knowledgeable professional, but also a personal caretaker. The pharmacists on their part would be motivated to extend their competence and better their skills. "Galen Help" could also favor the cooperation among primary caregivers, specialized facilities and regional medical services, in order to obtain a better partnership between diagnostic-therapeutic paths and Hospital-Territory continuity, all this to avoid path fragmentation and improve care for the patient.

As a working model development for the project and its better qualification, an agreement has been lately signed between the UNIAMO FIMR onlus, UPFARM and SIMMESN (Italian Society for Metabolic Diseases and Neonatal Screening), whose specialists work in the major Italian Pediatric Hospitals. The SIMMESN, as in the agreement, undertakes to spread the "Galen Help" project all over the Specialist Centers thus creating a professional synergy with the adhering pharmacies. The estimated model is operatively represented by the synergy between the Rare Diseases Associations, the Hospital specialists and the accredited pharmacies, collecting the local patients' prescriptions and undertaking to prepare the medicine according to the established standards of the partnered University Center. In the meantime, the adhering pharmacies agree to support the rare diseases patients with all their help both for bureaucratic and practical problems. At present, beyond two-hundred pharmacies, located all over the Italian territory, are adhering to this project by putting at the disposal of the Rare Diseases associations, their professionalism and technical abilities.

The project's target is to create a pharmacies' net all over the European Union, giving a rational and clear service for the Galenic medicines' preparation, useful to the rare diseases patients. On that subject it would be necessary to create, inside the European Community, a specific coordinating Committee, so to establish the project's application fields then trusting its production to the Specialist Centers and the involved pharmacies. One of the project's critical points might be the poor consideration from the political world, not able to grasp all the effective saving advantages for each of the Healthcare Systems in the European Countries and the great care towards the Rare Diseases Patients.