EURORDIS
Rare Diseases Europe

C O N T R I B U T I O N

EURORDIS' RESPONSE TO THE CONSULTATION REGARDING COMMUNITY ACTION ON HEALTH SERVICES

30 January 2007

Introduction

EURORDIS - the European Organisation for Rare Diseases – is pleased to send its contribution to the open consultation regarding Community action on health services. EURORDIS represents more than 270 rare disease organisations from 32 countries, 19 of which are EU member states, thereby reflecting the voice of an estimated 30 million patients affected by rare diseases in the European Union. Rare diseases are those diseases affecting less than 5 out of 10,000 citizens.

In the context of the current debate on health services, EURORDIS encourages mobility of patients as much as mobility of health professionals, data, samples and expertise. At the same time, EURORDIS promotes the provision of high quality healthcare close to where patients live. Even though patient mobility allowing access to healthcare in other countries is very important, it has to be limited to key moments of the development of the disease, such as diagnosis, requesting a second opinion and important medical interventions (surgery, transplantation and other invasive medical interventions).

For this paper, EURORDIS gathered input from its members as well as the outcomes of a long-term reflection process mainly on the issue of Patient Mobility and cross-border provision of some health services, without tackling the permanent presence of a service provider and the temporary presence of persons.

Before addressing the questions of the Commission's Communication, it is important to underline that - from the answers of our members - the three main areas of concern for rare diseases patients are the following:

- The financial burden of healthcare obtained abroad and the uncertainty of the level of reimbursement when returning to the home country;
- The language barrier which appears to be a potential deterrent to patient mobility; and
- The lack of information on where the most appropriate healthcare is available abroad and how to get it.

Question 1: Current impact of cross-border healthcare

In 2004-2005, EURORDIS conducted a survey on diagnostic delay, in collaboration with INSERM (EurordisCare2[1]): 5,980 questionnaires were analysed, concerning 8 rare diseases (Crohn's disease, Cystic fibrosis, Duchenne muscular dystrophy, Ehlers-Danlos syndrome, Marfan syndrome, Prader Willi syndrome, Tuberous sclerosis, and Fragile X syndrome), and including patients from 14 EU countries (Austria, Belgium, Denmark, Finland, France, Germany, Ireland, Italy, Netherlands, Poland, Portugal, Spain, Sweden, United Kingdom), one accessing country (Romania) and Switzerland. On the specific issue of patient mobility, the survey showed that:

- In half of the countries, more than 1.8 % of rare disease patients had to travel to a different country to get an accurate diagnosis;
- In half of the countries, more than 24.6 % of rare disease patients had to travel to a different region to get the diagnosis;
- Diagnosis disclosure forced 1.5% of rare disease patients to change country of residence and approximately 1 to 5% to change region of residence.

Given the fact that rare diseases are specific in lack of experts and difficulty of diagnosis and treatment, the information mentioned above is consistent with the frequently quoted figure stating that 1% of the total population travels in order to get healthcare in a country other than the one of affiliation.

The main conclusions to be drawn from these figures are as follows:

- The number of rare disease patients travelling abroad (in order to get the right diagnosis) is real and represents a high number of consultations, given the total number of patients living in Europe with a rare disease (between 25 and 28 million citizens);
- Patient mobility from country to country does not represent an overwhelming figure such as to put in danger the overall budget of national health systems;
- Mobility from region to region, within the same country, shows that regional inequalities are also an important concern to take into account in the reflection process on health services. An excessive regionalisation of health could have serious consequences. Even though "interregional mobility" falls beyond the scope of the current Communication, EURORDIS wishes to underline that this represents an additional problem for rare disease patients.

More specifically, we can present the following country-specific figures on the proportion of patients who had to travel (abroad and not abroad) to get an accurate diagnosis: in half of the countries, 1.8% of the patients had to travel to another country to obtain their diagnosis, 24.6% had to travel to another region, and 72% did not need to travel at all.

[1] http://www.eurordis.org/article.php3?id_article=454

- **The countries where patients travelled the most to <u>another country</u>** were: Romania (6%), Italy (5.5 %), Spain (4.8 %), Ireland (2.5) and Poland (2.2 %);
- The countries where most patients had to travel to **<u>another region</u>** were: Romania (49.2%), Finland (47.2%), Poland (45%), Belgium (44%) and Ireland (35.4%);
- **The countries where patients <u>travelled the least</u>** were: Austria (84.9 %), Portugal (83.7%), Spain (81.9 %), Sweden (82.2%) and France (78.9%).

The establishment of European Reference Networks of Centres of Expertise is of primary importance in the issue of patient mobility across borders. However, it is hoped that the obligation for patients to travel will remain the exception, and that everything will be put into place for patients to benefit from the highest level of proximity care. Rare disease patients do not wish to become « patient-travellers ».

Question 2: the need for legal certainty - Legal clarification and practical information

2.1 Private or public coverage

Procedures and conditions for allowing rare disease patients to get healthcare abroad shall apply equally to both private and public insurance/healthcare coverage systems.

2.2 Delays

Given the lack of sufficient knowledge on the natural history of most rare diseases, the consequences of delays cannot be accurately evaluated. Nobody can currently say what is a “reasonable delay” as opposed to an “undue delay” in the context of rare diseases. There is no measurable “due delay” for having the confirmatory diagnosis of a severely debilitating, life-threatening rare disease, and there is no measurable “reasonable delay” to start a treatment or to undergo surgery. Limited knowledge on most rare diseases does not allow the majority of healthcare professionals to precisely assess the consequences of delaying the beginning of a treatment or the date of an intervention. Patients are often left alone with their decisions and have to bear the responsibility of making difficult decisions for themselves or on behalf of their loved ones. When one considers that even doctors do not know how long one can wait before undergoing surgery, how could designated administrative authorities evaluate whether the delay is “reasonable” or “undue”?

How will rare disease patients ever be able to prove that the delays encountered "at home" are not "medically acceptable"? In most cases consensus treatments and reference literature do not exist for rare diseases; it is thus impossible to measure the "acceptability" of a delay.

Example: Is it "medically acceptable" that an 18 month old child is made to wait 6 months before getting an accurate diagnosis for his/her condition and appropriate healthcare? Who can assess the "acceptability" of the risks in terms of worsening and potential irreversibility of his condition due to the delay, as well as the risks for his/her parents to have another affected child?

2.3 Care not provided in the country of affiliation

Legal certainty is also needed when patients purchase care in a different country from their country of affiliation and claim reimbursement in the latter. This can happen for example when the "home" healthcare provider does not offer the type of care that is sought. The level of reimbursement cannot be calculated in the country of affiliation in such a case; patients could be asked to request prior authorisation on the following conditions:

- The response is fast
- The patient can appeal if the response is negative
- The patient does not have to prove that the care he/she is seeking does not exist in his/her country of affiliation
- The patient is given a clear indication of the level of reimbursement that he/she will be entitled to when returning home
- The patient is informed exactly about the level of the cost of the needed health care services or pharmaceutical product

Examples:

i. A patient wishes to have a specialised biological examination that is not provided at home. He needs to travel abroad to give a tissue sample. When he receives the results and the bill, he claims reimbursement from his healthcare system. At the moment, he may be refused reimbursement on the basis of the non-classification of the examination and the absence of a reimbursement decision. He will have to pay out of his own pocket.

ii. A patient needs a pharmaceutical product that is not authorised in her country of affiliation, but is authorised in another Member State. Instead of travelling abroad to purchase the product, an "import authorisation" could be signed by national authorities and an authorisation of reimbursement given. In the absence of reimbursement decision, she will have to pay our of her own pocket.

2.4 Distinction between hospital and non-hospital care

Legal certainty is an absolute necessity concerning the definition of the central terms of "non-hospital care" and "hospital care". This clarification is urgently needed to

allow smooth implementation of patient mobility and cross-border healthcare. These definitions must be agreed at the European level to guarantee consistency in all the Member States, who currently diverge on the definitions and the way to distinguish hospital from non-hospital care. Therefore, to avoid inequalities, a European harmonisation is needed for the definition of hospital care.

In addition, the relevance of the distinction between hospital and non hospital care can be questioned, as illustrated by the Muller-Fauré case ruled by the ECJ (non-hospital care). In this case, the cost of dental treatment was much higher than the cost of the hospital care for Ms Van Riet. For Ms Fauré no prior authorisation was needed, whereas Ms Van Riet should have made a request for prior authorisation. The reason for prior authorisation is to limit financial risks for healthcare systems; however, the risk may be higher for non-hospital care expenses.

2.5 Conditions for prior authorisation

If the obligation for prior authorisation remains in future legislative texts, then the conditions for obtaining it should be defined explicitly, otherwise legal uncertainty will persist. Eurordis suggests EU decision-makers to establish a system in which proving that care is not available in a country of affiliation and that better quality care can be obtained in another country is not the responsibility of the patient. Because of these difficulties, requiring prior authorisation as it is currently conceived may result in obstacles to patient mobility. Positive or negative responses to a request for prior authorisation should be provided within 30 days, with the provision for an accelerated procedure in emergency cases or an *"a posteriori authorisation"* implying reimbursement.

A swift appeal procedure should also be implemented for cases where patients are not satisfied with the response to their request for prior authorisation.

2.6 Prior authorisation

For rare disease patients, requiring prior authorisation is a long, fastidious and discriminatory procedure. In fact, decisions by authorities in charge of granting authorisation are often more arbitrary and inaccurate for rare diseases than for common diseases because of the widespread lack of knowledge. In the best case scenario, complementary expertise delays the process and in the worst case scenario no complementary expertise is requested and the response is inappropriate. The decision of granting authorisation is taken either at the national or the regional level, often after consulting technical committees or advisory health professionals. Only in a few Member States are delays for taking these decisions mentioned and only one[2] Member State (Denmark) indicates the existence of an emergency procedure with very short delays and the possibility to give an *a posteriori* authorisation. The procedure for prior authorisation is often too complex, too long and

[2] DOCUMENT DE TRAVAIL DES SERVICES DE LA COMMISSION : RAPPORT DE SYNTHESE - APPLICATION DES REGLES DU MARCHE INTERIEUR AUX SERVICES DE SANTE MISE EN OEUVRE PAR LES ETATS MEMBRES DE LA JURISPRUDENCE DE LA COUR SEC (2003) 900

arbitrary. In the case of rare diseases, patients should be exempted from obligation to request prior authorisation or exempted from waiting for a response from the administration: after 15 days, in the absence of a response, it could be deemed to be positive.

2.7 Practical information

Eurordis would welcome the establishment of an easily accessible and user-friendly database of institutions providing specialised treatment and healthcare for rare diseases patients in the EU. The institutions listed should be recognised as providers of treatment and healthcare of a quality assured standard; patients using their services should be fully reimbursed.

The EU Rare Diseases Task Force is currently dealing with these issues; Orphanet is going to publish the list of EU Centres of Reference for rare diseases.

Question 3: Advance payment

For rare disease patients, it is particularly difficult to advance funds when travelling abroad is needed for healthcare reasons, because the cost of care required for diagnosis and treatment of rare diseases can be very high.

Furthermore, rare disease patients are in most cases not independent enough to travel without the assistance of another person, often a member of their family (typically a parent will accompany a sick child). Therefore, in addition to the healthcare expense itself, one must add the travel costs for two persons and the loss of income of the accompanying member of the family during the duration of the trip.

Another issue is that the level of reimbursement in the country of affiliation may be very disappointing compared to the actual cost incurred for the intervention performed abroad. The difference between these two amounts will have to be borne by the patient and/or his family.

When low reimbursement and expenses due to the trip are taken into account, only rich families will be able to afford patient mobility without putting their personal budgets at serious risk. Given the fact that having a rare disease patient causes an overall impoverishment of the family, most patients and families will not be able to afford patient mobility.

EURORDIS therefore strongly supports the establishment of a system where no advance payment is requested from the patient. Instead, the healthcare provider in the other Member State should send the bill to the healthcare system of affiliation.

Question 4: Redress and liability

EURORDIS believes that the liability remains with the healthcare provider.

For financial compensation, the question whether the healthcare system of affiliation or the healthcare system of the host country should compensate the harm, remains open.

Question 5: Compatibility with balanced medical and hospital services

There are two possible reimbursement systems: the “notification system” or the “authorisation system”.

- In the “notification system”, the patient simply notifies his healthcare system of his decision to purchase healthcare in another Member State. No authorisation is required. Reimbursement is limited to the amount that would be reimbursed in the country of affiliation. The consequence is that possible extra costs are borne by the patient.

- In the “authorisation system”, the patient requests prior authorisation. The decision can be negative or positive. A negative response should be fully justified in order to be opposable.

  In the case of a positive outcome, the costs are fully reimbursed by the healthcare system of affiliation, even if they are higher than the home ones. In this system, all costs are reimbursed provided that care has been previously authorised. In order to limit the delay for patients, a request for prior authorisation - and therefore the reimbursement - should be considered as accepted if the request is not answered negatively within 15 days.

  It has to be underlined that a different approach is needed in emergency situations. It is of course impossible for a patient to wait 15 days when he or she is facing an emergency, which can happen often in the case of chronic diseases.

Whether patients simply notify their decision to go abroad or whether they ask for prior authorisation, Eurordis asks that reimbursement from authorities in the country of origin of the patient covers the totality of the cost paid by patients when purchasing healthcare abroad.

Question 8: European action

As one of the basic principles underlying the reflection process leading to the creation of this document, EURORDIS wishes to indicate that rare disease patients feel strongly that the European Community should take action in health-related matters. According to the principle of subsidiarity, "*the Union does not take action (except in the areas which fall within its exclusive competence) unless it is more effective than action taken at national, regional or local level. It is closely bound up with the principles of proportionality and necessity, which require that any action by the Union should not go beyond what is necessary to achieve the objectives of the Treaty*"[3]. It is therefore important to recall that Article 152 of the Treaty clearly defines these objectives in the area of public health, as follows: "A high level of human health protection shall be ensured in the definition and implementation of all Community policies and activities. Community action, which shall complement national policies, shall be directed towards improving public health, preventing human illness and diseases, and obviating sources of danger to human health".

Given the legal basis for Community action in the field of Public Health combined with the principle of subsidiarity, Eurordis believes that health inequalities between Member States and the way to overcome them through - among others - a robust system allowing true patient mobility for all, represent an area of shared Community competence. Community action shall complement national policies while at the same time promoting co-operation and co-ordination between Member States towards a common goal: the highest level of Public Health for all European citizens in the whole of the Union.

It is essential for the European Commission to increase its effort to compare health outcomes and indicators and improve its communication strategy on health inequalities. In fact, Eurordis believes it is important that the Commission adequately informs the European public opinion on existing health inequalities in the EU through relevant media.

Question 9: tools to tackle the different issues related to health services at EU level – Community legislation

EURORDIS advocates for the adoption of a Community policy that would create an appropriate and sustainable environment for the establishment of European Reference Networks of Centres of Expertise (ERNCE) on rare diseases. Centres of reference facilitate the definition of multi-disciplinary treatments, access to a second opinion on diagnosis, therapy and medical/surgical interventions.

[3] This definition is taken from the glossary of terms relating to European integration and institutions, as well as the activities of the EU.

The benefits of European Reference Networks of Centres of Expertise for the rare disease community are as follows:

- For patients: identifying, comparing and choosing between relevant providers, in a context where expert centres are needed;
- In terms of medical progress: combining strengths and increasing resources to improve research on rare diseases;
- For healthcare professionals: exchanging information, multidisciplinary approach, high level of excellence allowing for the definition of treatment guidelines, optimisation of care;
- For healthcare systems: scale economy and rationalisation of expenses;
- For public health: conducting epidemiological studies, monitoring health indicators, rationalising health expenditure through a better use of care

One of the most crucial issues for rare disease patients and their families is the right to a second opinion, especially at critical moments of the development of the disease, such as the diagnosis phase, the decision to reassess the multidisciplinary therapy and important interventions (surgery, transplantations, etc.). While the necessary and most welcome debate on ERNCE is taking place at the European level, EURORDIS wishes to draw the attention to a potential threat to the right to a second opinion for rare disease patients. We believe that this matter should be discussed among relevant stakeholders to establish a system able to overcome this difficulty.

Among other issues related to the ERNCE, EURORDIS would like to draw the attention to the necessary cooperation and freedom of opinion of healthcare professionals involved in these Networks. For instance, if a rare disease patient benefits from care in the relevant Centre of Reference abroad, with due prior authorisation, will he/she then be entitled to consult another Centre for a second opinion abroad, under the same conditions (authorisation and reimbursement)?

In addition to the difficulty of seeking care in different centres abroad with the same authorisation and reimbursement conditions, another difficulty is the tendency of healthcare professional to retain patients in their own active files and to be reluctant to share information with other colleagues considered as potential competitors. This is why when an expert centre is part of an ERNCE, and if requested by the patient or his family, the centre should be obliged to share all data from the patient's medical files, share samples and accept full mobility of patients from one expert centre to another within the Reference Network - and this without any further administrative burden for the patient.

Another risk is that doctors and health professionals second expert colleagues and would not feel free to contradict a previous diagnosis or expert advice. We know from experience that experts can make mistakes, especially in the field of rare diseases. All conditions should be gathered to allow health professionals cooperating in the ERNCE to express their opinion freely and with the sole objective of defending patients' interests.

In this context EURORDIS proposes that authorisation to seek a second opinion is automatically renewed for rare disease patients on a three-year basis. This system would reverse the current approach where doctors (and care centres) think "this is my patient" to the patient-centred perspective "this is my disease" and "this is my life".

Generally speaking, EURORDIS promotes an EU Health policy where the norm is that rare disease patients have the right to travel abroad when they believe they need it, even if their doctor disagrees. Travelling abroad to seek healthcare is complex and burdensome enough to ensure that this route will only be chosen when absolutely necessary. As patients and/or parents of patients, we do not believe that this would create a large flow of patients wishing to travel abroad unreasonably. We also think that this approach would have a positive impact on national healthcare systems, pulling them towards increased quality and sharing of good practices.

Access to medical treatment and healthcare should be covered by a European Regulation directly applicable in every Member State, not through an EU Directive. Additional guidelines could be developed for specific situations and groups of diseases if necessary.

Conclusions

1. If prior authorisation is requested for reimbursement of care purchased in a country other than the country of affiliation, then a derogation should be possible for rare disease patients, as the authorisation process would be arbitrary, time consuming and discriminatory.

2. Derogations are in line with the rules of the European Court of Justice that already recognises cases where patients are exempted from prior authorisation, i.e. pensioned persons (Case C-326/00 Idryma Koinonikon Asfalisseon).

3. If derogation is not possible, a simple notification procedure should substitute the authorisation process. Alternatively, the authorising body could seek the opinion of the relevant patient organisation, when applicable.

4. Patients should be authorised to seek care in another country not only when care is not provided in their country of residence, but also on other grounds such as better quality care. It should not be up to patients to demonstrate either the absence of provision of care in their country or the better quality of care in the host country.

5. No advance payment should be requested from patients, as cost of care for rare diseases is often very high. Instead, the provider of care should see its expenses reimbursed by the healthcare system of affiliation.

6. The creation of sustainable networks of centres of reference could be an option to help identify care centres and compare them, as well as improve the quality of care. Second medical opinion should be easy to obtain, without precluding cooperation between healthcare professionals in various centres, and with automatic renewal.

7. If the distinction between hospital and non-hospital care should remain, then a standard definition should be provided. Conditions for obtaining prior authorisation should be explicit, providing the authorisation process does not delay access to care and does not discriminate nor misbalance the respective roles of the healthcare system and patients.