

**Consultation document on  
Organ Donation and Transplantation  
Response Form**

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**Contact details of institution submitting comments**

<b>Name of Institution</b>	EURORDIS European Organisation for Rare Diseases
<b>Address</b>	Plateforme Maladies Rares 102, rue Didot 75014 Paris France
<b>e-mail address</b>	<a href="mailto:fmacchia@eurordis.org">fmacchia@eurordis.org</a>

**1. This document describes the situation at European level in the area of organ transplantation, identifying the main problems. Are all the basic problems identified? Are the problems identified correctly described?**

In addition to the common problems encountered by all patients undergoing organ transplantation (risks linked to the anaesthesia and heavy operation itself, risks of graft rejection, heavy immunosuppressive therapy, etc.), EURORDIS would like to underline some issues more often encountered by rare diseases patients:

Organ transplantation is most of the time the only therapeutic option for a number of rare diseases patients, and we observe a higher proportion of organ transplantations in rare diseases patients than in the global population.

In the absence of effective medicinal products, patients affected by several rare diseases – such as lymphangiomyomatosis (lung), cystinosis (kidney), hemochromatosis (liver), Chron's disease (small bowel), keratoconus (cornea), Becker muscular dystrophy (heart), DiGeorge syndrome (thymus), syndactyly (skin) – need to go through life-saving organ transplantation. The period preceding the organ transplant is often particularly dreadful for rare diseases patients not only because they usually have more than one organ severely affected, but also because they need to take other drugs than the ones needed for the failing organ(s). Furthermore, in many cases, rare diseases patients do need multi-organ transplantation (e.g. patients with cystic fibrosis, alpha 1 anti-trypsin deficit, etc.). All these elements lead to an aggravated situation for rare diseases patients.

Interactions with other drugs: after the transplantation, rare diseases patients do have to continue their usual heavy treatment (usually just symptomatic), associating a high number of medicinal products, because of their complex conditions affecting generally more than one organ. The immunosuppressive regimen - needed for reducing the risks of graft rejection - may interfere with other drugs or other clinic manifestations of the disease (e.g. patients affected by recurrent infections whose conditions will be worsened by the necessary immunosuppressive therapy).

Living donors: because of the difficulties encountered by rare diseases patients to be accepted on the waiting lists for organ transplants (see point 4 below), there is an important percentage of transplantations performed on rare diseases patients from living donors and a high pressure on their family members. We feel that the issue of living donors has been lightly referred to in the Commission's consultation document, especially given the extreme complexity of situations encountered by health professionals and ethics committees in this field.

Influence of the disease itself on the exit of the transplantation: the good response and acceptance of the graft by the recipient may depend on the disease itself. This is the case, by instance, with the cornea transplantation in the bullous dystrophy macular type or in the keratoconus. Furthermore, the certainty of the disease recurrence on the graft, for example in the case of metabolic diseases, is also specific to certain rare diseases.

**2. The document also describes a number of actions oriented to tackle the main problems. Is there any other initiative that you consider useful?**

EURORDIS believes that concerning quality and safety issues, there is a real need for minimum common standards to be agreed and implemented at EU level.

An EU Directive, such as the ones on good clinical practices and on blood safety, would be welcome.

**3. The shortage of organ donors is being described as the main problem in the field. Do you think that EU action would have an added value? Do you think that the initiatives described in the document in this direction are sufficient? Are there any other actions that should be promoted at EU level?**

EURORDIS believes that there is scope for action at EU level on the issue of organ donation and transplantation and that a reinforced cooperation between Member States would be welcome, certainly in the areas of quality standards, traceability and availability of organs to better respond to the urgent and growing needs across the EU.

We feel that one potential area of cooperation has been overlooked in the Commission's consultation document: "awareness-raising" activities. In this field, supranational cooperation would most usefully lead to pan-European awareness-raising and information campaign directed to the European public opinion, thus ensuring the same level of information at EU level. We believe that patient organisations – at local, national and European level - should be involved in the information activities and that health professionals, in addition to public opinion, should be also targeted.

It has been referred by health professionals that when appropriately and pro-actively informed, families tend to agree to the donation of organs when the worst happens to one of their members. The notion of "informed consent", especially in countries where an explicit consent is lawfully needed, implies an education and information effort that can - and must - be legitimately performed at EU level.

**4. Accessibility to transplants varies widely in the EU. Do you think that the Commission should foster the coordination between Member States to improve the situation? Do you think that the initiatives described in the document in this direction are correct? Are there any other actions that should be promoted at EU level?**

Concerning accessibility, EURORDIS has been referred, by patients and health professionals in the transplantation field, that there is inequality in the access to organ transplants for rare diseases patients. The reason is simple – and understandable – but difficult to overcome and leads to dramatic situations for patients and their families.

Because of the low number of patients for each rare disease concerned by organ transplantation (with the exception of cystic fibrosis), there is a lack of validated transplantation indications for rare diseases. The existing internationally agreed standards do not allow for the validation of rare diseases indications, thus refraining access to organ transplants for rare diseases patients.

In order to be accepted on a waiting list for organ transplantation, the patient must have the “good indication”, which involves that there has been an assessment of the benefit that the patient with a particular disease would gain through the transplantation. This assessment is very difficult to perform when the disease involves a limited number of patients (this problem is similar to the issue of clinical trials with “small number” of patients eligible to participate to the trial).

Therefore, especially in the current context of organs shortage, a high number of rare diseases patients needing transplantation are not allowed to be inscribed on a waiting list for the organ they need in order to survive, because it has not been possible to assess the benefit.

Rare diseases do not follow the general trend of other diseases in the area of organ transplants: as stated in the Commission’s consultation document, while the “excellent result of transplants has multiplied the indications of these therapies”, this is not true for rare diseases where the evidence available today is not sufficient to assess the real benefit of a transplant for the recipients.

**5. The document presents the following three options for future EU policy on organ transplantation:**

**(1) Use of existing programmes only;**

**(2) Active coordination between Member States on organ quality, safety and availability;**

**(3) Minimum harmonisation on quality & safety, plus EU initiative on organ trafficking.**

**Which one of these options do you consider the most appropriate? Would you wish to modify / add / remove some of the contents included in the option? Please explain your reasons.**

We believe that a combination of (1) and (2) would be the most appropriate option: in the area of quality and safety, the definition of common standards would be very welcome and requires a Directive or a Regulation for its best implementation. EU initiative on organ trafficking also belongs to the Community sphere of action and has a strong legal basis.

In other areas, an active coordination between Member States would probably be the most appropriate option:

- In order to increase accessibility of organ transplants to rare diseases patients, efforts and evidence in this field should be gathered at EU level in order to overcome the obstacle of a too limited number of transplanted rare diseases patients. This would allow for the assessment of the real benefit, thus multiplying “good indications” also for rare diseases and reducing the current inequalities;
- As above-mentioned, in the area of “awareness-raising” pan-European Campaigning, a stronger cooperation between Member States, involving stakeholders, would be very beneficial and participate to the reduction of organ shortage;
- The sharing of best practices and exchange of information in the area of organ transplantation between health professionals. These events could be financed by a specific budget from DG SANCO.
- In order to facilitate the search for the closest possible match between donor and recipient, we believe that a central registry at EU level should be established on the basis of a voluntary cooperation between EU Member States, sharing common quality and safety standards.