European Commission
Health & Consumer Protection Directorate-General
Directorate C – Public Health and Risk Assessment
C2 – Health Information
Public consultation regarding European Action in the Field of Rare Diseases

Comments from Flemish Hospital Pharmacists

Question 1. Is the current EU definition of a rare disease satisfactory?

It would be good to create a subclass of ultra-orphan drugs (prevalence < 0,18 / 10 000 for example) as suggested in the British Medical Journal number 331 of October 29 2005 page 1016: "Orphan drugs and the NHS: should we value rarity?" by McCabe and co-workers.

As orphan drugs are always essential to the patient the WHO Model List of Essential Medicines needs an addendum on rare essentials: the Orphan Medicines Model List. This complementary list could improve access to these life-saving pharmaceuticals and encourage policy formation in their regard in emerging countries.

There is a great need for a list of orphan primary ingredients which are life-saving chemical compounds (with a "no drug use" label) with no pharmaceutical reference that need to be dispensed or compounded for the treatment of patients with ultra-rare diseases: monographs in the European Pharmacopoeia should be developed on these primary ingredients such as D-mannose, D-ribose, D,L-3-hydroxybutyrate sodium, fenfluramine, phenylbutyrate sodium, 3,4-diaminopyridine and dibutylsquaric acid. The compounding procedures also need to be standardized in an European Formulary for commercially unavailable preparations such as caffeine citrate, sodium benzoate and tri-sodium citrate injections as well as sodium benzoate, l-arginine and levocarnitine oral solutions.

A list of orphan drugs was already considered as such before the enactment of the EMEA Orphan Medicinal Product Regulation Act 141/2000 but they have yet to be confirmed as orphan drugs: Ammonaps, Benefix, Beromun, Cerezyme, Cystagon, Orlan, Quadramet and Vitravene.

Question 4. Should the European Reference Networks privilege the transfer of knowledge?

The Belgian Association of Hospital Pharmacists has established a database for orphan drugs, orphan primary ingredients and orphan medical devices. It is accessible to all (hospital)pharmacists and gives the commercial name, chemical name, pharmaceutical dosage form, local distributing company (telephone and fax-number, email address), and regional reimbursement data. There is also a discussion forum active among pharmacists on all subjects about orphan drugs such as availability, reimbursement, exchange of best practice and compounding procedures. The database turned out to be a very useful tool in the dispensing of orphan materials to patients with rare diseases and the forum is helping to spread the concept of orphan drugs and rare diseases among the profession.

Question 5. Should on-line and electronic tools be implemented in this area?

An electronic "metabolic intervention box" has been installed with clinical and pharmaceutical information (compounding procedures) on metabolic emergency interventions together with

information on the availability of these life-saving medications in other hospitals. The national poison control centres only give information on “environmentally acquired poison”. This on-line database was very useful in the dispensing of orphan medication to patients with metabolic disorders in emergency situations.
As we deal here with rare disorders the occurrence of side-effects is even more exceptional. For this reason we need to install an easily accessible on-line electronic platform for all parties involved that collects pharmacovigilance data connected to the correct use of these products.

Question 8. Do you envisage the solution to the orphan drugs accessibility problem on a national scale or an EU scale?

The aforementioned database of the Belgian Association of Hospital Pharmacists covers national as well as international distributors of orphan drugs, orphan primary ingredients and orphan medical devices because some companies do not provide their market-approved products to Belgium. For metabolic emergency interventions a network of tertiary-care, university teaching hospitals has been set up to ensure equal access to these life-saving substances that quite often need to be compounded.

Question 9. Should the EU have an orphan regulation on medical devices?

Definitely so. There is no reason at all to exclude these implants.

Question 10. What kind of specialised social and educational services for RD patients and their families should be recommended at EU level and at national level?

Compassionate use and medical need programs are already in place and does not need to be changed for orphan drugs as well as orphan primary ingredients and orphan medical devices.