



## Minutes

### First meeting of the “Task Force on Rare Diseases” (TFRD)

Luxembourg, 21 January 2004

#### Summary of the minutes

On the 21 January, a first meeting took place of a Task Force on Rare Diseases (TFRD), composed of former (1999-2003), ongoing (1999-2003) and future (2003-2008) SANCO project leaders, and experts from Member States. The TFRD is a reflection core group advising the EU Commission on how best to implement the EU Public Health Programme as regards Rare Diseases. The deliberations of this advisory group are routinely presented to the “Morbidity and Mortality Working Party (MMWP)”. Main outputs were the discussion on an appropriate coordination of work with DG Research (RTD) Rare Diseases initiatives, an exchange of information between project leaders not yet having had the opportunity to meet nor disseminate their work at the EU level, despite being funded from Community programmes, and the agreement of experts to participate in this group on a regular basis.

Finally, there was agreement to support a Commission/ Council Presidency conference to diffuse the results of the former Community Rare Diseases Programme (1999-2003), to take place in 2005 (see web for pre-announcement<sup>1</sup>) and an agreement on defining Rare Diseases Health Indicators in close collaboration with the concept and development of SANCO C/2 ECHI list<sup>2</sup> (European Community Health Indicators).

In addition, the meeting minutes and all related documents are published in due course on the SANCO website EUROPA.

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<sup>1</sup> [http://europa.eu.int/comm/health/ph\\_threats/non\\_com/ev\\_pre2005\\_en.htm](http://europa.eu.int/comm/health/ph_threats/non_com/ev_pre2005_en.htm)

<sup>2</sup> [http://europa.eu.int/comm/health/ph\\_information/indicators/indic\\_data\\_en.htm](http://europa.eu.int/comm/health/ph_information/indicators/indic_data_en.htm)

**1. Introduction** (John F. Ryan, SANCO C/2 Head of Unit and Dr Frédéric Sicard, Seconded National Expert)

The new Programme of EU Community action in the field of Public Health (2003-2008) aims to develop an integrated approach to protect human health and improve public health. It is based on three general objectives:

- To improve information and knowledge for the development of public health
- To enhance the capability of responding rapidly and in a coordinated fashion to threats to health
- To promote health and prevent disease through addressing health determinants across all policies and activities

Institutions, associations, organisations and bodies in the public health field are encouraged to submit projects for implementing specific priorities as defined in Work Plans by the Commission, on an annual basis.

Rare Diseases are one of the important priorities in the new Programme. As per Annex 2.3 of the new Programme, activities related to Rare Diseases include the development of strategies and mechanisms for the exchange of information and prevention. According to the Work Plan for 2004, the two main axes of actions are the exchange of information by using existing European information networks on Rare Diseases as well as the development of strategies and mechanisms for exchange of information and co-ordination at EU Community level to encourage continuity of work and trans-national co-operation. Furthermore, regarding Rare Diseases projects, DG SANCO C/2 prioritizes generalist networks, which centralize information on as many diseases as possible and not just a specific group or a single Rare Disease.

The financial envelope of the Programme for the period 2003-2008 is € 312 million. This amount is divided into the 'operational budget' (to award grants for projects and by issuing calls for tender) and to resources for technical and administrative assistance and support expenditure. The intention is to allocate the operational budget in a balanced way between the three objectives of the programme. There are 2 procedures available for applicants wishing to take part in the Public Health Programme, call for proposals and call for tenders. In the case of call for proposals, Programme Grants are restricted to a maximum of 80% of the budget.

The European Commission wishes to be supported in its task to implement the Programme of Community Action in the field of Public Health (2003-2008) by a Task Force on Rare Diseases, which will comprise experts suggested by Member States, EEA/EFTA and Candidate countries, leaders of projects funded during the previous programme on RD (1999-2003), some concerned project leaders from the previous Health Monitoring Programme (1997-2002) and leaders of RD projects funded during the new programme (2003-2008) as well as experts from International organisations (such as WHO-EURO and IARC), EUROSTAT concerned Task Forces and Core groups, plus concerned European Commission services.

Mr Ryan underlined the importance given to Rare Diseases, which is one of the priorities of the new programme. He indicated that Rare Diseases had been initially assigned to the second strand 'health threats', which was later judged inappropriate. This is the reason why Rare Diseases have now been moved to the strand 'Health information' and are linked in the annual work plans with the Working Party on 'Morbidity-Mortality' (MMWP).

This TFRD is so a sub-group of the MMWP which means that tasks and work developed within the TF should be in coherency with the MMWP general mandate.

In order to give more visibility to the programme, the final technical project reports will be published on the Europa website. The Commission would like so to improve the dissemination of information on Rare Diseases funded projects.

A new call for proposals will be published soon. Concerning RD proposals, it will be similar to the 2003 call. Interested applicants are invited to follow the terms of the call published on Europa.

The Commission has taken the initiative to organise a conference on Rare Diseases during the first semester of 2005, in Luxembourg during the Luxembourg presidency, in order to publicize the European achievements in the field (DG Sanco, DG Research, DG Enterprise, European Agency for the Evaluation of Medicinal Products, NGOs). It is expected that one of the applicants will propose to organise the event and will apply for the necessary funding in the coming call. The conference will be different from the one which will be organised in early 2005 in Sweden by the Karolinska Institute and the US National Institute of Health (NIH) which will have a pharmaceutical transatlantic and treatment forms, from information received to date.

DG SANCO will invite external reviewers to evaluate the proposals of the next call. The constitution of the panel of external experts is in process. It was questioned why this decision of using external experts is so late, when this has always been requested by Member States representatives. Mr Ryan pointed out the difficulty to identify experts with no conflict of interest.

After Mr Ryan has left the meeting, Dr Frédéric Sicard took over the chairing of the meeting.

## **2. Role of the Task Force (Dr Frédéric Sicard)**

Dr Sicard underlined the fact that Task Forces will have relatively few members comprising of dedicated experts on the field (e.g. Rare Diseases). Its size should be no more than 20 people (for reimbursement purposes). The participants were invited to declare whether they wanted to be an active member of the Task Force or observer. The draft list of volunteers is attached.

All project leaders of the newly financed projects will systematically be invited to join the Task Force. It is also desirable to invite representatives of other projects from HMP<sup>3</sup> being related to the Task Force objectives, such as PERISTAT, CHILD, EUDIP, EUROCISS and EUROCHIP.

The Task Force will have to agree on its mandate to be submitted by the Commission (SANCO C/2).

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<sup>3</sup> Health Monitoring Programme – projects:

- PERISTAT: Indicators for Monitoring and Evaluating Perinatal Health in Europe
- Bold CHILD: Based on Live Data Child Health Indicators of Life and Development
- EUDIP: Establishment of Indicators Monitoring Diabetes Mellitus its Morbidity
- EUROCISS: Cardiovascular Indicators Surveillance set in Europe
- EUROCHIP: Health Indicators for Monitoring Cancer in Europe

Tasks must be also thought in relationship to and complementary with DG Research activities (Unit F2). It is also necessary to contribute to the MMWP efforts to define relevant health indicators. The Task Force should propose indicators that are specific, consistent and robust in close collaboration and coherency with ECHI (European Community Health Indicators) concept and short list developed by SANCO C/2.

Comments from participants:

- It is necessary to build on previously funded projects for the knowledge acquired not to be wasted (*This is why SANCO C/2 has included former project leaders as full members of the TFRD*).
- The development of an extension to the existing classification and coding system is urgently needed. Many specialist networks are working in this area. It would be highly desirable to establish links with these groups and to work in close partnership with WHO. Classification of new entities should also be included.
- Many cancers are rare forms of cancer but they should not be forgotten (*This is why SANCO C/2 has invited EUROCHIP project leader and IARC to be members of the TFRD*)

**3. Presentation of the work in the former Rare Disease Programme and the new 2003 projects** (see contributions)

24 projects were conducted under the former Programme of EU Community action on Rare Diseases (1999-2003). These can be found on the Europa Website: [http://www.europa.eu.int/comm/health/ph\\_projects/rarediseases\\_project\\_en.htm](http://www.europa.eu.int/comm/health/ph_projects/rarediseases_project_en.htm)

The total budget allocated for this programme was 6.5 million Euros. Strengthening or creation of major established European Networks such as ORPHANET, EUROCAT and EURORDIS was achieved through certain of these projects.

- a. Presentation of ORPHANET (phases 1 to 4): database of Rare Diseases and orphan drugs
- b. Presentation of EUROCAT (phases 1 to 3): European surveillance of congenital anomalies
- c. Presentation of EURORDIS (phases 1 to 3): European Patients Organisation on Rare Diseases
- d. Presentation of NEPHIRD (phases 1 and 2): network of public health institutions on Rare Diseases
- e. Presentation of SCN: European network on the epidemiology, pathophysiology and treatment of Severe Chronic Neutropenia

**4. Presentation of the 2003 and 2004 Work Plans and RD in the framework of the Public Health Programme** (see Introduction)

**5. Presentation of the Rare Diseases activities in DG Research/Unit F2** (Mary Fitzgerald) (also see contributions)

DG Research contribution to research support for Rare Diseases is evident in the Fifth Framework Programme (34 projects constituting €45 Million of funding) and in

the Sixth Framework Programme as per the Council Decision 30/09/2002 'to pool Europe's research resources for tackling rare diseases'. As an illustration topics open for funding in the first two calls were displayed.

Questions and comments from participants:

- What is the real strategy of DG Research? Is there only room for very large projects? Answer: *The fundamental shift from 5FWP approach to 6FWP approach aims to achieve a critical mass in terms of scientific output through the funding of much larger consortia and providing greater flexibility with significantly larger budgets. Currently 75% of funding would be geared towards the larger NEW INSTRUMENTS known as Integrated Projects and Network of Excellence. 25% of the budget would be retained to fund the smaller traditional type instruments, similar to those in 5FWP. Thus the very large projects are going to be a hallmark of the 6FWP*
- It was clear from the discussion that attitudes varied towards the new approach, some people declaring a preference for the old system, others realistic about the importance placed on the NEW INSTRUMENTS by the Commission and eager to capitalise on potential funding opportunities.

Close collaboration of stakeholders in the field of Rare Diseases, in order to increase the available budgets, was proposed.

## **6. Nomination of "Task Force leader" and " Task Force deputy leader"**

The Commission defined the Task Force leader and deputy as the "contact persons" between SANCO C/2 and TFRD. The Task Force leader and deputy will participate to all co-ordination meetings and represent the TFRD at the NWPL (Network of Working Party Leaders) and the NCA (Network of Competent Authorities). Dr Sicard proposed Ségolène Aymé (ORPHANET) to be the Task Force leader and Helen Dolk (EUROCAT) to be the Task Force deputy. As the nomination process was not discussed in advance and as no one had the opportunity to introduce his/her candidacy, a participant suggested that the leadership should be reconsidered in one year's time, which was accepted by the two nominees. Ségolène Aymé suggested to work mostly by conference calls as the number of formal meetings will be 2 per year. The group asked the Commission to organise the next Task Force meeting before July 2004. Dr Sicard agreed to explore further this option<sup>4</sup>.

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<sup>4</sup> For SANCO C/2 internal reasons, the second TFRD meeting will only take place in October 2004