AIM
ASSOCIATION INTERNATIONALE DE LA MUTUALITE

AIM response to the DG SANCO consultation on "Rare diseases: Europe's challenges"

Brussels, 14 February 2008

AIM welcomes the document of consultation on rare diseases, which is really well documented and gives a precise and interesting overview of the topic. But AIM has some remarks which are included within the questions to be answered.

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

- Yes, AIM considers the existing definition of rare disease (RD) in the EU adopted by the Community action programme on RD 1999-2003 in principle satisfactory. However a definition based only on the prevalence does not give any evidence yet on the development and the severity of the illness. Many of these diseases can be life-threatening or lead to chronically illness, but it is not always the case. This has to be clearer.

Question 2: Do you agree that there is a pressing need to improve coding and classification in this area?

- Yes, AIM agrees with this need and support the idea of a working group on Classification and Codification of RD, acting as Advisory Working Group to the WHO in the ICD revision process. In correlation with the answer to question, it should be checked, if it is possible to distinguish between rare diseases and very rare diseases, and to give more criteria on the consequences of a disease.

Question 3: Can a European inventory of rare diseases help your national/regional system to better deal with RD?

- Yes, the members of AIM reckon it and AIM think the European Commission should provide financial support for this activity through the Public Health Programme. A European inventory can improve transparency. The extent to which it can be helpful depends of its concrete structuring.

Question 4: Should the European Reference Networks privilege the transfer of knowledge? The mobility of patients? Both? How?

- AIM believes that centre of references on RD are a key element and that the information among them should be shared through European Reference Networks which have to privilege transfer of knowledge, as well as the mobility of patients with RD. The latter could enhance health pathways for patient by sending them to European Reference Centers when they do not exist in their country or by using telemedicine.
  The questions of the principles of authorisation and reimbursement of the costs of the cross-border care (including travel costs) are solved by the European regulation 883/2004 or through structured cross border agreements between countries concerning RD.

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- A European Quality certification of such Reference Centers (RC) or Centers of expertise should be developed.

- AIM deems that the Commission should continue funding on the long run the European Reference Networks and the Members States (MS) have to keep on funding their RC.

Question 5: Should on-line and electronic tools be implemented in the area of RD?

- Yes, AIM supports the importance of such tools. It would perhaps be desirable to have a central electronic data base on e.g. study situation.

Question 6: What can be done to further improve access to quality testing for RD?

- Given the large number of tests needed and the impossibility for a single country to be self-sufficient in the provision of testing, AIM supports the cross-border flow of patient material and testing and the need in this area to develop clearly stated, transparent, "EU agreed standards and procedures". As this matter concerns cross-border health, AIM supports also the need "for bridging regulatory differences among countries in confidentiality practices**,** reimbursement, sample transport and storage and certification of laboratories". Quality assurance and certification of reference laboratories should be fostered.

Question 7: Do you see a major need in having an EU level assessment of potential population screening for RD?

- Yes, and AIM deems that coordination in this area is needed to allow countries taking evidence-based decisions. Concerning the necessary criteria for the assessment, the international accepted scientific standards also require, that the disease intended to be screened, has to be an important health problem.

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

- AIM believes that the solution to the orphan drugs access problem is to be envisaged a European level, taking into account that the reimbursement decision have to be done at national level.
  Indeed AIM considers that the major hurdle to unequal orphan drugs access is the problem of affordability. Concerning the prices of orphan drugs, AIM members request companies to disclose key elements of their cost components, in order to have a rational basis for price negotiations. Such 'transparency' of the components (transfer price) underlying the final price is of utmost importance for economic optimisation between supplier, payer and patient across Europe.
  Secondly, the current orphan drugs market authorization system could be refined in order to better regulate the monopolies created by the system.

  AIM outlines that the administrative delays beyond the 180 days legal limit in the availability of authorised orphan drugs, should not be considered as a hurdle, but more as a need of a country to have more time to assess the safety and the relative effectiveness of the drug considered for safety reasons.

- AIM underlines that the networking of pharmacovigilance data on orphan drugs should be improved.

- AIM agrees that the Commission should present every two years a report to the Council and the Parliament identifying the bottlenecks to access to orphan drugs like delays, marketing, access, reimbursement, prices, etc., **as well as eventual new indications for orphan drugs, the existence of national plans, use of referral procedures to**

centres of expertise and the impact of the orphan drugs on the health expenses for each country. AIM aggress also that the Commission makes proposals on the necessary legislative modifications in order to guarantee equal access to orphan drugs throughout the EU.

- AIM asks for a re-evaluation of Regulation (EC) No 141/2000, particularly on the need of a systematic evaluation after 5 years of every orphan drug.
  On the basis of the experience with products authorised under the orphan drug Regulation, and taking into account the tremendously high prices requested for these products as well as the enlargement of the authorised therapeutical indications of these products, AIM would like to make the following two recommendations:

  - To organise a **systematical review** at the end of the fifth year of concerned orphan medicinal products. The orphan drug product sponsor should have the obligation to comply a review file (based on the five years experience) which has to be submitted to the EMEA in particular to the COMP committee. This information should be made available to the Member States. Who else would be better placed than the sponsor himself to provide data on the 5 years experience related to the original designation criterion (prevalence – return on investment - significant benefit).

  - AIM has serious concerns about the usefulness to organise a two step procedure. The Regulation adopted was clear: a review should take place after a period of 5 years. If the conditions for the designation as orphan drugs are not any more fulfilled, the market exclusivity period should be reduced to six years. In our opinion the two step procedure, looking if new arguments could be put forward would deny art. 8(1) of Regulation No 141/2000.

- Concerning primary preventive measures for RD, AIM agrees that action in this field should be the topic for a debate at EU level aiming to determine for which RD primary preventive measures may be successful.

- AIM highlights the role of the EU to promote the exchange of best practices among MS.

Question 9: Should the EU have an orphan regulation on medical devices and diagnostics?

- No, AIM believes that at this time there is not enough information or evidence on the need of an orphan regulation on medical devices and diagnostics.

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?

- AIM considers that at national level, specialised social services on RD should be integrated into existing local social services for dependency/long term care, by training the adequate people, in order to spend efficiently the scarce resources devoted to dependency where the need will grows dramatically in the future.

  At European level, AIM thinks that the EU should foster the exchanges of best practices on this area, through peer reviews.

  In addition to the national support, the European Commission should provide financial support for specialised services through the Public Health Programme and the Disability Action Plans.

- Regarding health technology assessment of orphan drugs, AIM highlights the need to assess the relative effectiveness of orphan drugs and their re-evaluation after 5 years, as mentioned above. A coordinated approach to this issue by MS is of course necessary.

- As far as compassionate use is concerned, AIM thinks that the funding of compassionate use should coming from the enterprises.

Question 11: What model of governance and of funding scheme would be appropriate for registries, databases and biobanks?

- In relation with governance, AIM believes that the resources should be accessible upon agreed rules being national or European depending of the funding sources, with board composed by different stakeholders (public sector, industry, patients, consumers, social health insurers, ..).

  AIM considers that all these databases, registries, etc.. should remain transparent and accessible to all.

- AIM reckons the need of funding by community level for European registries, databases and biobanks, while national funding efforts should complement the EU research funding for national infrastructure.

Question 12: How do you see the role of partners (industry and charities) in an EU action on rare diseases? What model would be the most appropriate?

- AIM believes also that in addition to the orphan drug regulations, where the pharmaceutical industry is involved, academic research in preclinical development should be supported by the public sector, the EU and charities.

  AIM supports the public-private partnership (industry and universities, ..) to the evaluation of these drug candidates in the field of RD, should be fostered and funded by the EC, the public sector and the charities, in order to intensify research in innovative biotechnological research (monoclonal antibodies, cell and gene therapy, and enzyme replacement therapy) as well as classical therapeutic research based on the search for active chemical compounds. But for health insurance association it would not be acceptable to finance research and development by public money while private companies on the other hand take all the profit for pharmaceuticals, therefore the PPP should be well regulated to avoid such situation and monopolies.
  The Drugs for Neglected Diseases Initiative (DNID, www.dndi.org) is a good example of a new way for developing drugs, where the PPP model is effective and where at the end the price of the drugs is also very affordable for the patients and the society.

  Lastly AIM thinks also that independent academic clinical trials should be supported at European and national level on the model of what as been done so far in Italy, France and Spain and these efforts should be coordinated to ensure enough patient participation.

- AIM supports acutely the confidentiality and the protection of patient's privacy, based on the EC Data Protection Directive.

- AIM agrees that need to continue the EU FP6-supported ERA-NET project coordinating the funding policies for RD of seven countries and the need to invite additional MS invited to join this initiative, as a successful solution to the fragmented research in RD.

Question 13: Do you agree with the idea of having action plans? If yes should it be at national or regional level in your country?

- Yes, AIM deems that having action plans on RD is a need. They should be developed at national level, as the particularity of rarity of the RD and implemented at regional level.

AIM supports the idea of European guidelines for the elaboration of action plans for RD, which are already part of the Public Health Programme as a priority for action.

- Concerning the patient organisations, whose role is very important, AIM highlights that they have to be funded by public authorities and EU programmes, instead of the pharma industry to become and remain become really independent.

Question 14: Do you consider it necessary to establish a new European Agency on RD and to launch a feasibility study in 2009?

- No, AIM does not see any evidence on the added value of a New Agency on RD, but AIM supports the idea of the creation of an EU Advisory Committee on RD, replacing the existing task force on RD.

About AIM

The Association Internationale de la Mutualité (International Association of Mutual benefit societies) (AIM), created in 1950, brings together 45 national federations of autonomous health insurance and social protection bodies in 28 countries, all operating according to the principles of solidarity and not for-profit orientation. They provide coverage against sickness and other social welfare risks to more than 170 million people, either by participating directly in the management of compulsory health insurance, by providing voluntary health insurance or by delivering directly health care and social services through own facilities.

AIM's goal is to defend and promote, at international and European level, the social values and basic principles shared by its members: access to health care as a fundamental right, solidarity and non-exclusion as essential means to ensure this access to quality health care for all, irrespective of health status or financial capacity to pay; finally, autonomous management and non profit orientation as guiding principles for health insurance based upon the needs of citizens.

AIM endeavours to voice concerns and ideas raised within the sphere of non-profit health insurance institutions in the EU. AIM positions, requiring validation through its own statutory decision-making process, do not commit its individual member organisations. Therefore, AIM involvement does not detract from its member organisations taking dissentient views.

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