www.theibta.org

We are an alliance of brain tumour support, advocacy and information groups around the world, including brain tumour patients and caregivers, researchers, scientists, clinicians and allied health professionals who work in the field.

greater collaboration ► greater knowledge ► greater hope

EUROPEAN COMMISSION
HEALTH & CONSUMER PROTECTION DIRECTORATE-GENERAL
Directorate C - Public Health and Risk Assessment
C2 - Health information

PUBLIC CONSULTATION
RARE DISEASES: EUROPE'S CHALLENGES

RESPONSE BY THE INTERNATIONAL BRAIN TUMOUR ALLIANCE (IBTA)
16 December 2007

Introduction: The International Brain Tumour Alliance (IBTA) seeks to be an alliance of the support, advocacy and information groups for brain tumour patients and carers in different countries and also includes researchers, scientists, clinicians and allied healthcare professionals who work in the area of brain tumours. (See www.theibta.org) We have a particular focus on Europe and regularly participate in and exhibit at scientific conferences held in Europe e.g. Warsaw (2007), England (2007), Barcelona (2007), Paris (2005), Vienna (2006).

In constructing our response the IBTA sought comment from all the European-based brain tumour-related groups with which it is in contact.

We have sought to respond to the EC public consultation in the manner indicated in the discussion document.

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

Yes. During 2006-2007 the IBTA commissioned a statistical study by the Central Brain Tumor Registry of the United States (CBTRUS) to identify the world incidence and number of primary malignant brain tumours. The results are available here: http://www.theibta.org/uploads/file/Statistics.htm Based on GLOBOCAN 2002 figures they identified a world incidence for males of 3.7 per 100,000 per annum and for

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females of 2.6 per 100,000 per annum. The European incidence was 6.1 and 4.3 respectively. We do not know the reason for the contrast between the European and World incidences. The CBTRUS also calculated incidences of 3 and 2.1 for primary non-malignant brain tumours. We are aware that there has been an uneven development of cancer registries in Europe and the IBTA calls for more resources to be devoted to this problem. We would hope that from such attention more accurate statistics about CNS tumours will emerge, including their grade, type, and location. Although the CBTRUS was unable to accurately calculate the incidence of brain metastases (often called "secondaries") they are known to be frequent. Metastases to the brain arise from a cancer elsewhere in the body.

Question2: *Do you agree that there is a pressing need to improve coding and classification in this area?*

We cannot answer for other diseases but the classification of CNS tumours is based on a WHO classification system which is continually being reviewed by specialists who work in this area. In regard to that classification system various countries are endeavouring to have so-called benign brain tumours included in statistical measurements of brain tumour incidence because, unlike benign tumours in other parts of the body, such tumours within the CNS can have a lethal effect.

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

We believe this information would arise from the development of a better cancer registry system in Europe.

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

The information networks referred to in 4.2 should also apply to patients and caregivers.

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

Serious attention should be devoted to the promotion of on-line resources, including email discussion groups (not necessarily web-based groups), so as to permit greater communication between patients and caregivers affected by an RD. Responsible authorities should endeavour to facilitate the participation in these groups of health professionals who are knowledgeable about the RD with which the discussion group is identified.

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

Screening or anticipatory investigations are not appropriate for CNS tumours in the absence of symptoms. Diagnostic and genetic tests in relation to other RDs should only be conducted under strict ethical, legal and moral guidelines.

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

Not relevant to CNS tumours.

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

There are obvious inefficiencies and delays between countries in approval of the therapies relevant to CNS tumours but not necessarily confined to those that have been designated as orphan drugs.

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

Yes. Future therapies which may benefit brain tumour patients include convection enhanced delivery (CED) mechanisms to the brain, and a device worn on the head and designed to impart radio waves to attack the tumour. These are not medications and may come under the description of devices.

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?*

Attention has to be given to the incorporation within health technology assessments of ways of factoring in non-economic criteria as they relate to a specific RD. We emphatically agree with the comment that economic evaluation is only one element of the decision-making process.

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

No comment.

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?*

Industry could be more supportive in developing the evidence base necessary for official approval of certain medications for use in a palliative setting which, unfortunately, is very relevant to CNS primary malignant tumours. We refer particularly to the IAHPC list of Essential Medicines for Palliative Care.

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, if they lead to greater integration of effort, less duplication, and have a report-back mechanism in-built into their application.

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

No comment.

Denis Strangman, IBTA Chair
Kathy Oliver, IBTA Secretary