European LeukemiaNet
Network Coordinator: Prof. Dr. R. Hehlmann
Network Manager: Dr. S. Saußele
Medizinische Fakultät Mannheim
Ruprecht-Karls-Universität Heidelberg
Pettenkofer Str. 22
D-68169 Mannheim (Germany)

Fon +49 621 383 6931
Fax +49 621 383 6932
E-Mail: nmc@leukemia-net.org
Homepage: www.leukemia-net.org

European Commission
Health and Consumers Directorate General
Directorate C – Public Health and Risk Assessment
Ms. Patricia Brunko, head of Unit Pharmaceuticals
Sanco-pharmaceuticals@ec.europa.eu

09/05/11

Response to the Public Consultation on the Concept Paper Revision of the "Clinical Trials Directive" 2001/20/EC

Dear Ms. Brunko,

I am writing on behalf of patients with fatal incurable diseases which have been left out of consideration by the clinical trials directive 2001. My position is based on 40 years clinical care for these patients and is supported by the patients´ representatives (EPF) and paediatricians (SIOPE) as present in Brussels on March 31, 2011, by the CML advocates, by the patients´ groups within the European LeukemiaNet and the German Competence Network on Leukemia, of which I am the Coordinator, by the German Competence Net lymphoma and by other organizations such as EHA and ESF.

The European LeukemiaNet (ELN) is a network of excellence of European clinical trialists on leukemia and related diseases and of their interdisciplinary partner groups. Its goals are the prolongation of life and the improvement of cure rates of patients with leukemia across Europe. This is primarily achieved by treatment optimization. Leukemia is a mostly fatal disease and requires, if acute, rapid action.

ELN, EHA and ESF will submit their own positions separately, but agree in the following points:

1. The risk of the disease should be considered when evaluating the risk of therapy.
   A patient who is confronted with a more than 90% probability of dying of his/her disease within a short period of time will happily accept a therapy with a mortality risk of 20%; if this therapy offers a 40% chance of cure or of significant prolongation of survival.

2. The instrument treatment optimization that has been successfully applied over 3 decades to help patients with fatal incurable diseases such as the leukemias should be treated differently from other trials.

   Many fatal incurable diseases such as the leukemias and many cancers are rare diseases which are frequently uninteresting for investments by pharmaceutic companies. The only chance of help these patients have is by treatment optimization. Treatment optimization studies use registered medicines in different combinations and sequences, non-drug strategies such as radiation or transplantation, and may include off-label use of registered medicines.

3. The rules for carrying out academic clinical trials should become simpler, less bureaucratic and less expensive.

For more specific comments I refer to the attached answers to your consultation questions. I thank you for the opportunity to contribute to the further development on the clinical trials directive and will be available for further questions if needed.

Sincerely yours,

Prof. R. Hehlmann
Coordinator European LeukemiaNet
Coordinator German Competence Network Leukemia
Coordinator German CML Study group