Public consultation on the Advanced Therapy Regulation.

In the Netherlands, heads of stem cell transplantation laboratories in academic centres and Sanquin and hospital pharmacists of academic centers decided in 2009 to join forces and form a new ATMP working group. The group was formed to enhance the interaction between these two groups of professionals, each with their specific knowledge related either to the processing of cells or the pharmaceutical aspects of the manufacturing process. Recently, the Flemish speaking Belgian colleagues joined this initiative. Although there are differences in the regulatory framework in Belgium and the Netherlands, all members work together to increase the safety and perform translational research for clinical trials.

Our response:

2.3. Hospital exemption.
The Advanced Therapy Regulation empowers Member States to authorise the use of advanced therapy medicinal products in hospitals for individual patients in the absence of a marketing authorisation. The so-called hospital exemption provides for flexibility to address the situation of individual patients; however, a too large application of this exemption may discourage the application for marketing authorisations.

Expertise on the manufacturing of cellular therapy products is present in academic centers since the early eighties, when the first stem cell transplants were processed and infused. Since then, new products were developed and our knowledge on processing of cellular therapy products, including ATMPs, increased considerably. The academic centers have a unique and crucial role in the development of second/third generations of ATMP products and are the pioneers for new (personalized) treatment options for individual patients. All new ATMP products are currently in clinical trials, in our view the best option to study safety and efficacy of these products in the treatment of patients.

1. Most of the ATMP products are being manufactured for individual patients and therefore, there is no need to apply for a marketing authorisation. At the end of a clinical trial, ATMPs that have been tested and proven to be safe and effective should be produced as a “standard of care” product in licensed facilities.

2. The new clinical trials regulation will enhance the number of international clinical trials. The classification of products in these clinical trials will in part be harmonized by this new regulation, but in our view it is also essential to harmonize the classification of ATMPs produced and applied under the HE.

3. Finally, ATMPs that are substantially manipulated require high level manufacturing expertise. In clinical trials, these products are manufactured in specialized centers and are allowed to cross border. When an ATMP is recognized as a “standard of care” product, we think that the academic centers with the appropriate licensing and expertise should be able to send products across borders.

2.5. Scope and adaptation to technical progress.
The Advanced Therapy Regulation applies to gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products.
Please provide your views on the scope of the Regulation and in particular as to whether the

scope should be modified to take account of technical progress.

Non-profit academic centers have a unique and crucial role in the development of ATMP products and are the pioneers for new (personalized) therapy options for individual patients. Their goal is to test ATMP products in clinical trials for the benefit of patients suffering from chronic and life-threatening diseases. The incentive is mostly not marketing authorization and therefore does not fit the traditional marketing-directed clinical trial strategy of pharmaceutical companies. In order to facilitate innovative developments of second/third generation ATMPs for the European patient population, adjusted regulation should be provided to non-profit academic centers.

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