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Quality of gene therapy products: EDQM listens to stakeholders' needs

The EDQM is the Directorate of the Council of Europe responsible for supporting the basic human right of access to good quality medicines and health care in Europe. With its European Pharmacopoeia (Ph. Eur.), the EDQM sets requirements for the quality of medicines, including those in the field of Gene Therapy Products (GTPs). Activities in the field started in 2000 with the creation of the Ph. Eur.'s GTP Working Party whose work led to the publication of general chapter *5.14 "Gene transfer medicinal products for human use"* in 2006. In March 2018, the Ph. Eur. Commission decided to reactivate this Working Party and entrusted it with the revision of general chapter *5.14* to take into account more recently elaborated pharmacopoeial texts such as general chapter *5.2.12 "Raw materials of biological origin for the production of cell-based and gene therapy medicinal products"*, and to assess the need to revise other general chapters or elaborate new Ph. Eur. texts related to gene therapy to include the latest developments in this fast-moving area.

The EDQM also provides the technical secretariat for the network of Official Medicines Control Laboratories (OMCLs) of Europe, which are responsible for monitoring the quality of medicines across Europe. This network of more than 70 public laboratories supports European regulatory authorities in ensuring the quality of medicines, including GTPs. In order to prepare for the developments in the field of GTPs, the Network set up a dedicated Gene Therapy (GT) Working Group in 2008. Its work programme focuses on the development and validation of relevant analytical methods, as recommended in general chapter *5.14* of the European Pharmacopoeia, with the emphasis on vectors used for GTPs that are already available on the market, as well as those that are in advanced clinical trials (e.g. adeno-associated viral (AAV) vectors, retroviral/lentiviral vectors (RV/LV), plasmid vectors).

The Ph. Eur. GTP WP and the OMCL's GT WG held a joint meeting in September 2018 also using this opportunity to gather feedback from their stakeholders. On this occasion, a conference was hosted by the Italian OMCL (*Istituto Superiore di Sanità*) in Rome on 27 September 2018. Attracting 52 participants from numerous regulatory authorities, industry, academia and associations, the conference was opened by Dr Carlo Pini, Head of the National Centre for the Control and Evaluation of Medicines and Dr Karl-Heinz Buchheit, Head of the Department Biological Standardisation, OMCL Network and HealthCare (DBO), EDQM, both of whom emphasised the importance of achieving harmonised quality requirements in this rapidly evolving field. The day continued with a short introduction from the EDQM staff about current activities of the EDQM and its working groups in the field of GTPs, followed by regulatory talks from the perspective of both Europe and the United States. Industry representatives and associations were then given the opportunity to present their wishes and vision as regards the role that the EDQM should play in the coming years in the field of GTPs.

These presentations and the ensuing discussions gave rise to a number of conclusions and recommendations (details can be found here). Defining quality requirements for complex, innovative, patient-specific products available in minute quantities and administered to patients with life-threatening conditions who are in need of urgent treatment is highly challenging. But, as one participant pointed out, "as long as the science is good, it is acceptable". There was a general agreement that, despite the variety of products concerned, the EDQM could and should contribute to the convergence of some of the testing methods used and to provision of reference materials.

During the following day, both the Ph. Eur. GTP WP and the OMCL's GT WG continued their discussions separately in order to ensure appropriate follow-up for their respective activities. Both groups closed their meetings with specific action plans for future activities in the field.

Contact: Caroline Larsen Le Tarnec, Public Relations Division, EDQM, Council of Europe
Tel.: +33 (0) 3 88 41 28 15 - E-mail: caroline.letarnec@edqm.eu

Note for the Editor: Further information is available on the internet site **https://www.edqm.eu/**
The EDQM is a leading organisation that protects public health by enabling development, supporting implementation, and monitoring the application of quality standards for safe medicines and their safe use. Our standards are recognised as a scientific benchmark worldwide. The European Pharmacopoeia is legally binding in member states[1]. Similarly, the EDQM develops guidance and standards in the areas of blood transfusion, organ transplantation and consumer health issues.

[1]There are thirty-nine members of the European Pharmacopoeia Commission: *Austria, Belgium, Bosnia and Herzegovina, Bulgaria, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Republic of Moldova, Montenegro, Netherlands, Norway, Poland, Portugal, Romania, Serbia, Slovak Republic, Slovenia, Spain, Sweden, Switzerland, "the former Yugoslav Republic of Macedonia", Turkey, Ukraine, United Kingdom and the European Union.*

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