

Challenges in the quality control of Gene Therapy Products

Let's work them out together!



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Why is the EDQM/Council of Europe involved in Gene Therapy Products?

■ The EDQM is the Directorate of the Council of Europe in charge of supporting the basic human right of access to good quality medicines and health care in Europe. With its European Pharmacopoeia (Ph. Eur.), the EDQM establishes official standards that apply to the quality control of medicines, including those in the field of Gene Therapy Products (GTPs), e.g. Ph. Eur. General Chapters 5.14. "Gene transfer medicinal products for human use" or 5.2.12. "Raw materials of biological origin for the production of cell-based and gene therapy medicinal products". The EDQM works on the standardisation of testing methods and in the future will also work on the development of reference standards for the quality control of GTPs.

■ Furthermore, the EDQM also acts as the technical secretariat for the network of Official Medicines Control Laboratories (OMCLs) of Europe, which are responsible for the surveillance of the quality of medicines across Europe.

Why and how are OMCLs involved in GTPs?

■ The OMCLs, a network of more than 70 public and industry-independent laboratories, support European regulatory authorities in ensuring the quality of medicines, including GTPs. Their role is to monitor marketed medicines in line with current legal requirements and support licensing authorities across Europe, e.g. in the review of data prior to clinical testing and in the assessment of Marketing Authorisation Applications. The OMCLs work in full transparency and respect the confidentiality of the information provided by the medicine manufacturers and producers.

■ Early engagement with European regulators and their OMCLs is important for manufacturers and producers, particularly manufacturers of GTPs, so that all relevant regulatory aspects can be taken into due account from the early stages of product development.

■ On the EDQM website, you can find more information on the activities of the General European OMCL Network (GEON) coordinated by the EDQM, as well as the list of the OMCLs which are members of the GTP Working Group (OMCL Network/Working group activities/Gene Therapy). More information can be found on the EDQM website: [go.edqm.eu/GTP](https://www.edqm.eu/GTP)

How do the EDQM and the OMCLs prepare for the market surveillance of GTPs?

■ GTPs are highly complex and innovative medicines that can act as a life-saving therapy for severe genetic disorders. GTPs also come with new and specific risks that will present challenges to OMCLs as regards their market surveillance task.

■ 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. This Working Group fosters collaboration between OMCLs working in the field of GTPs and encourages sharing of knowledge and technologies as a way of saving time and resources, increasing efficiency and promoting work in Centres of Excellence.

■ The GT Working Group meets once a year to discuss activities in the field and to review its work programme in line with the latest developments. This work programme focuses on the main vectors used for marketed GTPs, as well as those that are in advanced clinical trials, such as adeno-associated viral (AAV) vectors, retroviral/lentiviral (RV/LV) vectors, plasmids, etc. For each type of vector, the work focuses on the development and validation of relevant analytical methods, as recommended in general chapter 5.14 of the European Pharmacopoeia.

What about activities of the Ph. Eur. in this field?

■ The Ph. Eur. Commission decided in March 2018 to reactivate its own GTP Working Party and tasked it with the revision of General Chapter 5.14. "Gene transfer medicinal products for human use" to take account of 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".

■ The Working Party will also assess the need to revise any other general chapters or elaborate new Ph. Eur. texts related to gene therapy to include the latest developments in this fast moving area. In addition, they will participate in the revision of transversal texts elaborated by other Groups of Experts or Working Parties of the Ph. Eur., such as general chapter 2.6.35 "Quantification and characterisation of residual host cell DNA".

■ In line with the new policy of the Ph. Eur. Commission, experts from all around the world are welcome to participate in this work. You can find more information on how to become a Ph. Eur. expert on the EDQM website: <https://www.edqm.eu/en/join-network>.

How can the work of the EDQM, its Ph. Eur. and the OMCLs in the field of GTPs be supported?

■ Input and support from manufacturers of GTPs and interested parties is essential to establish appropriate methods and standards to ensure that the GTPs received by patients are both effective and safe.

■ The EDQM, its Ph. Eur. and the OMCLs stand ready to interact with all manufacturers in relation to activities on GTPs and rely on their support in providing study materials, as well as on their participation in activities aimed at defining the most appropriate state-of-the-art analytical methods for GTPs and relevant reference standards.



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