

REGULATORY ASPECTS OF MANUFACTURING AND CONTROL OF GENETICALLY MODIFIED CELLS

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Therapies based on genetically modified cells hold great promise for the treatment of various diseases with often limited treatment options. Last year, Strimvelis, consisting of CD34+ hematopoietic stem cells equipped with the correct version of the ADA gene for the treatment of the severe combined immunodeficiency condition ADA-SCID, has received marketing authorization in the European Union. Convincing clinical success has also been observed with recombinant chimeric antigen receptor expressing T-cells used to treat patients suffering from B-cell malignancies.

In respect to manufacturing and quality control, however, genetically modified cells are considered to be most complex medicinal products. Not only cell procurement, cultivation and genetic modification of the cells and their release testing needs particular consideration, also the vector used to deliver the modifying genetic sequence requires suitable design and proper manufacturing.

In this presentation, these fundamental steps in cell therapy manufacturing will be discussed from a regulatory perspective. Challenges regarding process consistency and manufacturing process comparability, as well as GMP expectations will be highlighted. Furthermore, a brief overview of the European regulatory landscape for cell therapies and novel developments in their regulation will be given.