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Implications of cGMP for CRISPR-Cas9 cellular therapy

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Many current therapeutic treatments are not able to address the underlying cause of a disease, alter its course, or reverse the damage that has already occurred. Cellular therapies offer the power of the human body to heal and regenerate itself. Regulatory precedents for cellular therapy products continue to evolve for a widening array of the product of types. An exciting new discovery of clustered regularly interspaced short palindromic repeat (CRISPR) technology when incorporated with cellular therapy may lead to the cure of many diseases. CRISPR-Cas9 offers the hope of a cure for various maladies including genetic diseases and cancers. The CRISPR-Cas9 medical cellular therapies involve removing cells from the body, modifying their DNA, and administering them to the patient. These modified cells are able to either replace or attack diseased cells. Medical cellular therapies are required to demonstrate quality, safety, and efficacy standards to obtain a marketing authorization. Medicinal cellular therapy products are regulated as drugs, devices, and biological products, which adds the regulatory requirement of manufacturing under cGMP conditions. With the high value of CRISPR-Cas9 source cell material, having ample amount for process-development and validation of the manufacturing processes is an industry challenge. Furthermore, limited shelf life and quantity of cells can complicate quality control testing and stability determinations. Defining critical quality attribute's (CQA's) for these products and developing assays for their potency are essential to the commercialization of these cellular therapy products. CRISPR-Cas9 source cells are characterized based on the presence of surface markers, size, and combinations of attributes associated with cell source and mode of action. Due to their ability to alter DNA, CRISPR-Cas9 cellular therapies offer the possibility to move beyond conventional disease treatment by addressing the underlying cause of disease, altering its course, or reversing the damage that has already occurred. The transitions from discovery, to research and development, to commercially manufactured products, brings the challenge of the regulatory requirements for incorporating cGMPs into the collection, production, and delivery of these products. These developments will allow for CRISPR-Cas9 cellular therapy to become increasingly available to patients and will offer new treatments and the hope to cure many diseases.

Biography

Buytaert-Hoefen obtained a Bachelor's degree in Psychology at the State University of New York at Binghamton and then went on to complete her Master's and Doctorate degrees in Neuroscience at the University of Colorado at Boulder. She completed two post-doctoral fellowships at the University of Colorado Health Sciences Center where she specialized in embryonic and adult stem cell research. She then entered the industry with a position as a Lead Scientist at Navigant Biotechnologies. After which, she accepted a position as a Consumer Safety Officer at the FDA, where she specialized in pharmaceutical inspections with an emphasis on biotechnology and sterile processing. Currently, as a Consultant at Parexel, she works closely with clients to develop and implement effective compliance solutions in accordance with client needs. She performs GXP audits, conducts laboratory data review including chemistry, microbiology, and data integrity assessments.

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