MANUFACTURING PROCESSES AND STRATEGIES FOR CELL AND GENE THERAPY PRODUCTS

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The development of novel, affordable and efficacious therapeutics will be necessary to ensure the continued progression in the standard of global healthcare. With the potential to address previously unmet patient needs as well as tackling the social and economic effects of chronic and age-related conditions, cell and gene-based therapies (CGTs) will lead the new generation of healthcare products set to improve global health and wealth. However, it is estimated that lot size requirements will exceed billions or even trillions of cells, a demand which cannot be supplied by current production methods. The difficulties associated with the translation of efficacious CGTs is not limited to developing our biological understanding of the cell, but rather encompasses engineering, biomanufacturing, regulatory, commercial and clinical challenges which must be addressed in parallel.

The CGT therapeutic modality, where the cell forms the basis of the product, is a paradigm shift for the biopharmaceutical industry. This talk will introduce some of the key challenges associated with the bioprocess development and consistent manufacture of CGTs that SMEs have, or will soon encounter, as they navigate through clinical trials.

An overview will be provided of the manufacturing processes and strategies employed to ensure the scalable and robust production of living cell-based products that maintain the critical quality attributes whilst minimizing manufacturing costs. Specifically, the role and opportunities afforded by single-use systems will be explored.

Additionally, this talk will outline some of the unique challenges associated with the large-scale production of cells intended for patient administration. This includes the diversity of source material for CGTs with respect to both cell candidates and variability in source material, the different treatment paradigms (autologous, allogeneic, haploidentical) and the need to produce complex biological products living cells which undergo little or no purification post-expansion. Finally, the need for single-use, customisable, controlled, flexible, cost-effective manufacturing platforms to accommodate the specific requirements of CGTs will be described.