

Gene Therapy: Triumphs, Challenges, and Future Directions



- 17th March, 2:00 PM CET
- Main Lecture Room, Kemijski Inštitut

Gene therapy stands as the culmination of over 150 years of scientific progress, tracing its roots to the foundational work of Gregor Mendel and Charles Darwin. This revolutionary approach to treating genetic disorders represents the convergence of genetics, molecular biology, and medicine, embodying the relentless pursuit of understanding and manipulating the fundamental building blocks of life.

The journey from Mendel's pea plants to today's cutting-edge gene editing techniques has been marked by both remarkable achievements and significant challenges. Recent years have witnessed tremendous advancements in gene therapy tools, including the refinement of viral and non-viral vectors. However, the path to clinical success has been complex, with obstacles ranging from vector efficacy and safety concerns to insufficient knowledge of disease mechanisms and a lack of reliable models and biomarkers. Despite these hurdles, the landscape of gene therapy is rapidly evolving. Breakthroughs in identifying causative gene mutations and common disease mechanisms are opening new therapeutic avenues. The advent of gene editing technologies and induced pluripotent stem cells has revolutionized our ability to create more accurate disease models and develop novel cell sources for regenerative medicine.

Examples of work on polyglutamine Machado-Joseph disease exemplify the potential of gene therapy in addressing brain disorders. Over two decades, we have developed promising technologies encompassing gene replacement, gene silencing, gene editing, and stem cell transplantation. These approaches not only offer hope for this specific condition but also serve as a model for tackling other neurological diseases.







With hundreds of therapeutic strategies in development and more than 20 approved products, gene therapy is poised to transform the treatment of monogenic diseases and cancer. However, high prices severely limit equitable access to patients worldwide. Approved products have been withdrawn or promising programs discontinued due to lack of reimbursement by healthcare systems or insufficient commercial interest. To remedy these shortcomings, initiatives are underway to guarantee universal access and find sustainable, financially acceptable alternative models.

The ongoing implementation of GeneT (Portugal) and CTGCT (Slovenia), two Centers of Excellence in Gene Therapy, along with the recently launched GeneH Excellence Hub, represents a significant step forward in addressing these challenges. These initiatives connect expertise across national borders to tackle bottlenecks in clinical translation, manufacturing scalability, and regulatory pathways. By fostering collaboration across borders, they aim to realize the full potential of gene therapy in addressing unmet medical needs while strengthening Europe's leadership in this transformative field.

This seminar will explore the current state of gene therapy, highlighting both its successes and ongoing challenges. By examining its historical context and recent breakthroughs alongside these collaborative initiatives, we aim to foster discussions on overcoming remaining barriers to ensure equitable access to life-changing therapies worldwide.



