The Personalised Medicine Network
Personalised Medicine Research Theme
Center for Genomics and Personalised Medicine

SEMINAR
Approaches to Personalised Medicine at Health

Gene therapy for primary immune deficiencies: From molecular diagnosis to gene edited stem cells

January 23rd 2023 at 14.00 – 15.30
Steno Diabetes Center AUH, Verdensrummet (A201-170), Aarhus Universitetshospital, Forum, Indgang A

Speakers:
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Abstract:
Next generation sequencing (NGS) has emerged as a powerful tool in the diagnosis of primary immune deficiencies. Primary immune deficiencies are a group of rare genetic disorders that result in a compromised immune system, making individuals with these conditions more susceptible to infections and other health complications. Traditionally, the diagnosis of primary immune deficiencies has relied on a combination of clinical presentation, family history, and immunological testing. However, the genetic basis of these disorders is often complex and can be difficult to identify using these approaches alone. NGS allows for the simultaneous sequencing of many genes or even an entire genome, enabling the rapid and accurate molecular diagnosis of primary immune deficiencies. This is crucial for identifying the specific genetic mutation responsible for the disorder and for determining the most appropriate treatment plan.
Once the genetic mutation has been identified, the CRISPR/Cas9 gene editing technology can be used to correct the mutation in the patient's hematopoietic stem cells, which give rise to all cells in the immune system. These gene edited stem cells can then be transplanted back into the patient, where they can give rise to a functional immune system. This approach has the potential to cure individuals with primary immune deficiencies. It represents a significant advance in the treatment of these conditions and has the potential to provide a permanent fix for the underlying genetic cause.
Overall, the combination of NGS and CRISPR/Cas9 gene therapy has the potential to revolutionize the treatment of primary immune deficiencies. It may offer a highly precise and effective approach for correcting the genetic mutations responsible for these disorders and for restoring the immune system to full function in these patients.

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Coffee and cake will be served after the seminar