Opinion Article

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Neurological Glycogen Storage Diseases from Gene Therapy to Enzyme Replacement Strategies

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Description

Due to their low occurrence, Rare Cancers (RCs), which make up more than 20% of cancer cases, present considerable research and therapy hurdles. When compared to more prevalent cancers, this leads to less than ideal results. RBTs represent 5%–10% of instances of uncommon cancer and present particular diagnostic challenges. For RBTs, the therapeutic potential of anti-cancer medications is still mainly unknown. Precision medicine in RBTs depends on identifying molecular changes in cancer-related genes and the pathways that link to them. potential treatments include monoclonal antibodies and small molecule inhibitors that target particular RBT-associated proteins. The goal of ongoing clinical trials is to identify subtypes, combination treatments and immuno-therapeutic drugs that are best suited for treating RBT biomarkers. This review sheds light on the current status of RBT biomarkers, possible therapeutic targets and potential inhibitors while addressing the difficulties in diagnosing, treating and researching RBTs. To enhance clinical results, uncommon malignancies require focus and creative solutions. In modest, phase 2 clinical trials, microbiota therapies that transfer fecal material from healthy donors to those with mild-to-moderate ulcerative colitis have demonstrated the ability to produce remission in approximately 30% of parti-cipants. Despite this significant accomplishment, the field still needs to identify the unique clinical niche for this new therapeutic modality within Inflammatory Bowel Disease (IBD) therapeutics, as well as capitalize on the insights obtained from these trials to move forward towards phase 3 clinical trials and drug approval. We outline the takeaways from previous research on microbiota therapies, ranging from defined *in vitro* products to full spectrum donor stool.

About inflammatory bowel disease

We examine the practical guidance these lessons offer on the planning of short-term research projects and future directions for the use of microbial therapies to the management of inflammatory bowel disease. If successful, microbiota treatments will offer a potent orthogonal strategy to increase the therapeutic ceiling for the large number of IBD patient non-responders and partial responders. It is acknowledged that the complement system regulates the immune system and tissue homoeostasis in addition to acting as a barrier against blood-borne infections. Dysregulated complement activity, however, is linked to inflammation and unwelcome or unresolving immune responses, which either cause or worsen the pathophysiology of a wide variety of autoimmune and inflammatory disorders. Despite the long-standing recognition of the benefits of targeting complement clinically, the approval rate of complement drugs has been rather low overall. Nonetheless, attempts to target complement therapeutically have been revitalized due to the efficacious treatment of atypical hemolytic syndrome and paroxysmal nocturnal hemoglobinuria by the humanized anti-C5 antibody eculizumab. A greater interest in introducing novel complement treatments into clinical practice has resulted from the identification of novel targets for drug development as a consequence of developments in complement biology and drug discovery methods. Although the growing number of licensed medications still mostly target rare disorders, there may be chances to increase the clinical targeting of complementary treatments for common diseases due to the huge pipeline of emerging therapeutic alternatives. A class of hereditary metabolic illnesses known as Glycogen Storage Diseases (GSDs) are defined by abnormal accumulation of glycogen as a result of abnormalities in glycogen metabolism in several tissues, with the liver, skeletal muscle and heart being the most commonly affected.

Pthophysiology of GSDs

Glycogen metabolism is critical for the brain's maintenance of numerous physiological processes and recent research has connected disorders of the Central Nervous System (CNS) to disruptions in this process. Because of this connection, a family of disorders known as neurological-GSDs was established. These diseases share deficiencies in the metabolism of neurological glycogen. The patients with non-GSDs display a range of clinical manifestations with a shared cause, necessitating customized treatment strategies from conventional GSDs. The patho-physiological underpinnings, genetic and pharmacological mechanisms of many n-GSDs have been clarified by recent study.

Furthermore, over the past ten years, there have been some encouraging advancements in innovative therapeutic tec-hniques, including as gene therapy, Substrate Reduction Therapy (SRT), Enzyme Replacement Therapy (ERT), small molecule medicines and gene therapy that target important components of glycogen metabolism in particular non-GSDs. Significant progress has been made in the preclinical stage toward potentially altering the course of the disease and enhancing patient outcomes. Here, we give a summary of current view points on non-genetically specified diseases highlighting recent developments in our understanding of their molecular basis, therapeutic advancements, important challenges and the need to further our understanding of the pathophysiology of n-GSDs in order to develop more effective therapeutic approaches that may provide patients with better treatment and long-term benefits.