PERSONALIZED GLUCOCORTICOID THERAPY FOR NEPHROTIC SYNDROME

Innovative diagnostic method based on genetic and epigenetic analysis

1. Unmet need

Nephrotic syndrome is an infrequent but severe disease mainly characterized by edema, massive proteinuria and hypoalbuminemia. The current mainstream therapy consists of glucocorticoids, which induce remission in 90% of the patients; within those patients almost 50% relapse and become steroid dependent. Steroid responsiveness is of major prognostic importance: patients with steroid dependence and resistance are at risk of more aggressive treatment and disease-related complications. Many efforts have been made to predict steroid response in children with the disease, however, to date, no definitive prognostic factor has been defined.

2. Technology

The main objective of the research is the development of an accurate and fast diagnostic test based on the study of the genetic and epigenetic mechanisms that lead to clinical response variability to glucocorticoids. The tool will allow to distinguish the different subgroups of patients (resistant, dependent and responsive) affected by nephrotic syndrome in order to personalize the glucocorticoid treatment. The tool will be developed considering the methylation status of two candidate genes, previously associated to glucocorticoid’s treatment response in leukemic patients, in combination with genome-wide analysis of other potential relevant sites and with miRNA analysis. The diagnostic kit will have clinical usefulness in order to develop an effective and personalized glucocorticoids therapy for patients, improving the treatment outcomes.

3. Main Advantages

The technology will allow the improvement of the therapy for nephrotic syndrome with advantages to the patients and the health system, leading to the development of personalized medicine strategies for this complex pathology. Moreover, the use of the diagnostic kit could be extended to other pathologies, such as rheumatoid arthritis and chronic inflammatory bowel disease, in order to identify genetic and epigenetic markers related to glucocorticoids treatment response. The reduction of treatment failures and the improvement of the quality of life of the patients will lead to a superior level of therapy for nephrotic syndrome, reducing costs related to problem of efficacy of the drugs and toxicity of the therapy.

4. Stage of development

The idea has been already validated in laboratory.
5. Intellectual Property

The technology is not protected by a patent.

6. Target Markets

The disease has a worldwide incidence of 1-7 per 100,000 in children and of 3 per 100,000 in adults. Personalized medicine is becoming one of the important trends in healthcare today and advances in medical genetics and human genetics have enabled a more detailed understanding of the impact of genetics in disease. Companion diagnostics are diagnostic tests based on biomarkers, that help to predict the response of the patients to the treatment. For that reason, this technology can be assimilated to this market. The companion diagnostics market is still underdeveloped and has enormous growing potential for further expansion. It is currently evaluated at roughly $2.7 billion and is expected to reach more than $7.6 billion by 2021.

7. Potential Partners

The potential partners are companies active in the field of advanced diagnostics or involved in the development of companion diagnostic tests for complex diseases.