



Research Project

Genome-guided drug identification: A randomized placebo-controlled trial on the influence of fampridine on working memory

Third-party funded project

Project title Genome-guided drug identification: A randomized placebo-controlled trial on the influence of fampridine on working memory

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Project start 01.05.2021

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Status Completed

Background: The discrepancy between the urgent need for improved therapeutic compounds and the lack of significant development of novel drugs illustrates the importance of pursuing new strategies aimed at identifying druggable targets related to psychiatric disease. Recent advances in large-scale genetic studies suggest that human genetic discoveries have the potential of translating into novel treatment targets for psychiatric conditions.

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Aim: To implement a drug repurposing strategy and perform a proof-of-concept clinical trial on the influence of a potassium channel blocker (fampridine) on working memory performance, an intermediate trait of psychiatric disorders.

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Methods: Guided by recent genetic findings in schizophrenia, we focus on those loci that point to genes targeted by currently approved drugs. As starting point serves the large schizophrenia genome-wide association study that provided genes robustly linked to the risk for the disorder. As intermediate filtering layer serves working memory performance, which is a well-established intermediate phenotype for schizophrenia. Finally, one repurposing candidate, fampridine, is selected and tested for its putative influence on working memory performance in the framework of a randomized placebo-controlled trial in healthy subjects.

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Expected value: We anticipate that the exponentially increased genetic knowledge of psychiatric disorders together with the use of biologically-informed phenotypes and appropriate data-mining methodology will be a starting point for the identification of novel drug targets and treatments, i.e. the very goal of the present proposal. Should the current randomized control trial turn out positive, it might open new avenues for the treatment of cognitive symptoms in psychiatric disorders with known and hopefully also new and specific potassium channel blockers.

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