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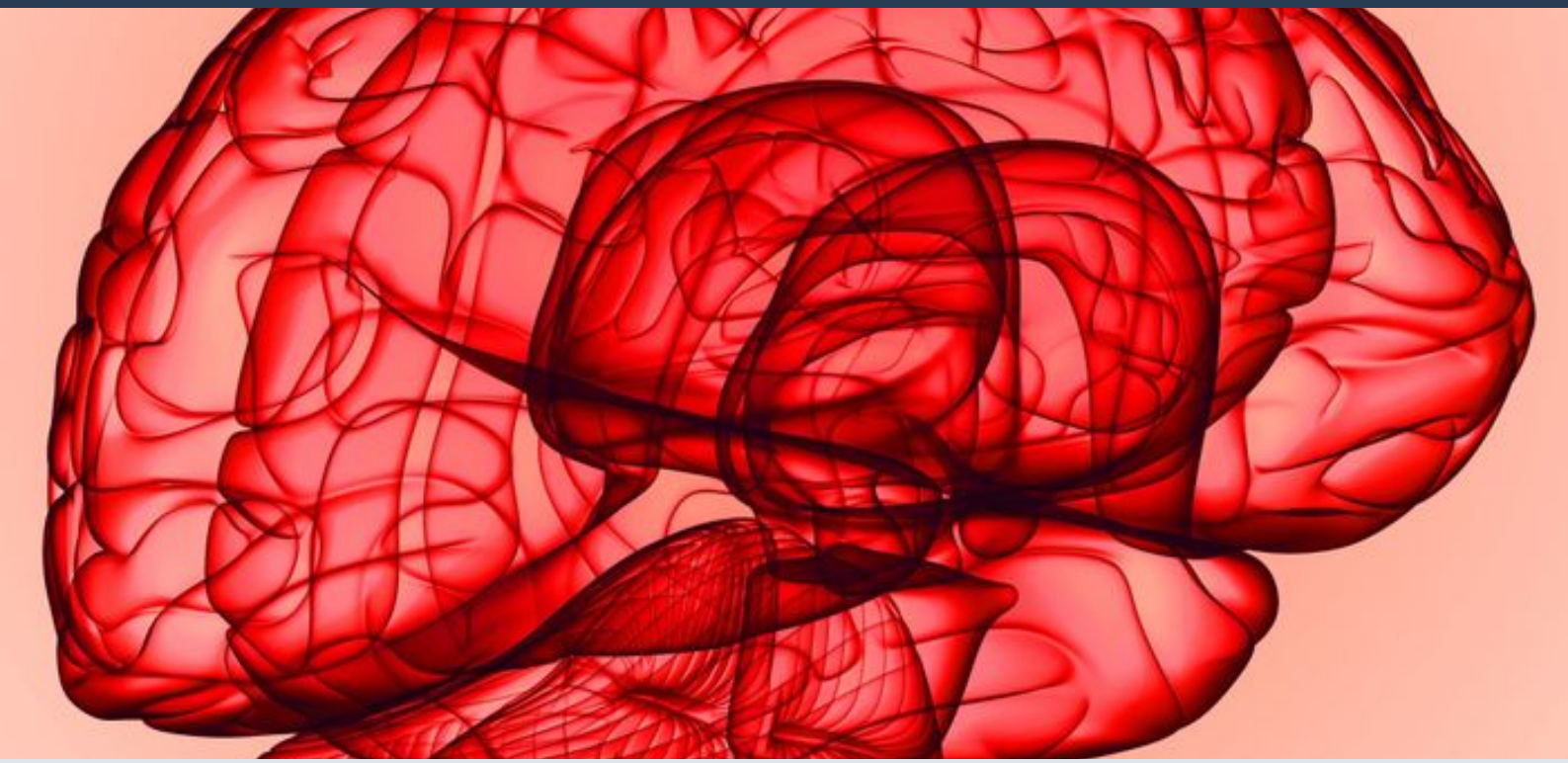
Human Brain-like Model System to Develop Treatment Solutions for Autism Spectrum Disorders

Gene-edited hybrid cerebral organoids from multiplex autism spectrum disorders families to investigate pathophysiological pathways

Reference: Brain-like Model



Source: https://stock.adobe.com/uk/images/red-3d-human-brain-model-3d-rendering/171524767?prev_url=detail

Seeking

Development partner

About **LMU Munich**

Ludwig-Maximilians-Universität München is the University in the heart of Munich. LMU is recognized as one of Europe's premier academic and research institutions. The LMU Munich community is engaged in generating new knowledge for the benefit of society at large.

Background

The pathophysiology of autism spectrum disorders is still poorly understood due to the marked heterogeneity and missing model systems. Reliable biomarkers are unknown so far. Diagnostic classification is therefore still based on subjective clinical impression and neuropsychological testing with unsatisfactory reliability and specificity. Moreover, these diagnostic procedures are highly resource- and time consuming. In addition, there are so far no mechanism-based treatment options tackling e.g. dysregulated genetic pathways or pharmacological interventions of core symptomatology. It is an unmet medical need to fill these essential gaps.

Tech Overview

To fill these essential gaps, LMU Munich researchers aim to study multiplex families consisting of patients with autism spectrum disorders (and other psychiatric disorders) and neurotypical controls using hybrid cerebral organoids, a recently established model system. The hybrid cerebral organoid model allows the team to investigate autism spectrum disorders-patient-derived cells vs. healthy control derived cells within the very same differentiated human cerebral organoid tissue.

This approach corresponds to a human brain-like model system ("mini brain"), which has strong potential to lead to the identification of fundamental pathophysiological changes, to unravel new autism spectrum disorders biomarkers, and to enable drug treatment response analysis.

Further Details

- The method of choice to knock-in large inserts via CRISPR | bioRxiv
- Gene edited fluorescent cerebral organoids to study human brain function and disease | bioRxiv
- ASD stem cell bank: Search CIRM | FujiFilm Cellular Dynamics, Inc. (fujifilmcdi.com)

Stage of Development

The hybrid organoid model system including CRISPR/Cas9 homology directed repair labelling of hiPSC lines, stem cell cultivation and sorting, organoid differentiation is fully established in the lab and is yet applicable to real life samples. In addition, access to the Munich Mental Health Biobank provides an excellent source for the validation of biomarker candidates in larger samples.

Benefits

- The researchers will deliver new highly reliable and cell-type specific autism spectrum disorders relevant molecular pathophysiological modifications

- In the hybrid organoid system differences in expression, subcellular localization, and cell-cell interaction can be allocated to specific cell types like neurons, astrocytes, microglia as well as to specific time points within the organoid development
- Current drug treatment strategies will be applied to the model system in order to define their impact on molecular pathway changes in autism spectrum disorders
- The suggested approach lends itself to individualized/personalized treatment options in the longer run

Applications

- The organoid model is a future concept in personalized medicine for autism spectrum disorders to identify molecular pathway aberrations in individual patients
- It enables pre-testing of specific drug combinations for a treatment the patient will benefit from as best as possible
- The technology is able to identify new biomarkers for more reliable and objective diagnostic classification
- The outpatient clinic database ensures the required recruitment volume of families and use cases.

For further information, please contact us.

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