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PRESS RELEASE

Breakthrough discovery offers hope for children with devastating epilepsy

Scientists identify new potential treatment from repurposed drug
An international team of researchers from the TreatKCNQ consortium, funded by the European Joint Programme on Rare Diseases, has uncovered an unexpected new use for an existing investigational drug that could bring relief to children suffering from KCNQ2-related developmental and epileptic encephalopathy (KCNQ2-DEE)—a rare and devastating form of childhood epilepsy.
The study, recently published in the British Journal of Pharmacology (DOI: 10.1111/bph.70119), reveals that JNJ-37822681, originally developed as an antipsychotic, also acts as a powerful opener of KCNQ2 potassium channels—the same mechanism targeted by the withdrawn anti-epileptic drug retigabine. JNJ-37822681 significantly reduced seizure-like activity in both patient-derived brain cells and animal models of epilepsy.

"This discovery brings us a huge step closer to an urgently needed treatment for children like ours."
—Quote from Aila and Oliver Coulman, parents of child with KCNQ2-DEE.

Hope through repurposing
The new findings are the result of a large-scale drug screening effort to find new uses for drugs that are already in advanced stages of clinical development. JNJ-37822681 emerged as a top candidate for activating KCNQ2 channels in human neurons—including those derived from children with KCNQ2 mutations. Importantly, JNJ-37822681 **lacks the safety issues** that led to the withdrawal of retigabine, the only other drug ever approved with this same therapeutic action.

From lab to clinic – the next hurdle
The research team confirmed the drug's effects in both neurons from KCNQ2-DEE patients, where it restored normal electrical activity, as well as in two established seizure models in mice.
While JNJ-37822681 was shelved by the drug originator for any further commercial development over a decade ago, the drug had already been successfully tested in hundreds of patients in clinical trials for safety and other neuropsychiatric indications. Most of these data are not available in the literature and the public domain. The investigative team is thus currently in a dialog with the drug originator to access and reference existing clinical data which will help to expedite clinical development of JNJ-37822681 in epilepsy.

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"We urgently need access to clinical data from past studies of JNJ-37822681 to help support further development and help us to design safe and efficient trials to test its potential in children with KCNQ2-DEE.", said the authors.

Why it matters

Epilepsy affects more than 50 million people worldwide. For families living with KCNQ2-DEE, seizures often begin in the first days of life and are accompanied by severe developmental delays. Currently, no precision medicine is approved for this condition. JNJ-37822681 could change that—offering the first hope of a targeted, effective, and safe treatment.