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Global Pharma News

FDA approves Roche's Evrysdi tablet as first and only tablet for Spinal Muscular Atrophy (SMA)

Roche announced that the U.S. FDA has approved a new Evrysdi® (risdiplam) tablet for people with spinal muscular atrophy (SMA). The 5 mg tablet, which can be swallowed whole or dispersed in water, offers a non-invasive disease-modifying treatment. This approval, based on bioequivalence study results, ensures that the tablet provides the same efficacy and safety as the existing oral solution. This new formulation offers flexibility and convenience, especially for daily activities such as working, traveling, and education. It is suitable for individuals two years and older who weigh more than 20 kg (44 lbs). The Evrysdi oral solution will remain available. Roche leads the clinical development of Evrysdi in collaboration with the SMA Foundation and PTC Therapeutics.

AskBio Receives FDA Regenerative Medicine Advanced Therapy designation for Parkinson's disease investigational gene therapy

AskBio Inc. announced that the investigational gene therapy AB-1005 for Parkinson's disease (PD) has been granted Regenerative Medicine Advanced Therapy (RMAT) designation by the U.S. FDA. This designation could expedite the development of AB-1005, which aims to slow disease progression and improve motor outcomes in PD patients. Preliminary clinical evidence, including 36-month Phase Ib data, showed that AB-1005 was well tolerated with no serious adverse events and indicated potential benefits in motor outcomes. The RMAT designation provides enhanced access to FDA guidance and support. AB-1005 is currently in Phase II clinical trials, with additional study sites expected to open soon.

Common drug shows promise against rare type of heart disease

A research team at the University of Arizona College of Medicine - Tucson discovered that an osteoporosis drug might counter a rare genetic mutation causing dilated cardiomyopathy (DCM). DCM weakens cardiac muscles, leading to life-threatening complications. Genetic mutations account for 30-40% of DCM cases, but no specific therapies exist. The team used

FDA-approved drugs to repurpose a treatment for rare mutations, focusing on the K210del mutation. They identified that the osteoporosis drug risedronate corrected the protein shape to normal and restored heart function in animal models. Clinical trials are planned to assess risedronate's efficacy in treating DCM in humans. This approach could also be applied to other rare genetic mutations.

NUS Medicine pioneers ground-breaking technique to deliver drugs directly to the brain

Scientists at the Yong Loo Lin School of Medicine, National University of Singapore (NUS Medicine) have developed a technique to deliver therapeutic molecules directly to the brain, bypassing the blood-brain barrier (BBB). Led by Dr. Haosheng Shen, the team used genetically engineered Lactobacillus plantarum (Lp) bacteria to produce and release therapeutic compounds through a nose-to-brain pathway. This method targets the olfactory mucosa, allowing localized and sustained release of medication, enhancing brain bioavailability. Preclinical studies showed that the modified bacteria successfully reached and accumulated in the brain, reducing appetite, lowering body weight gain, and improving glucose metabolism. This approach could be used to treat neurological conditions, including neurodegenerative diseases. The next research phase involves optimizing dosing regimens and conducting human clinical trials.