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ASIA EDITION

USFDA gives Orphan Drug Designation and Rare Paediatric Disease Designation to Aptamer developed in Hong Kong

23 December 2024 | News

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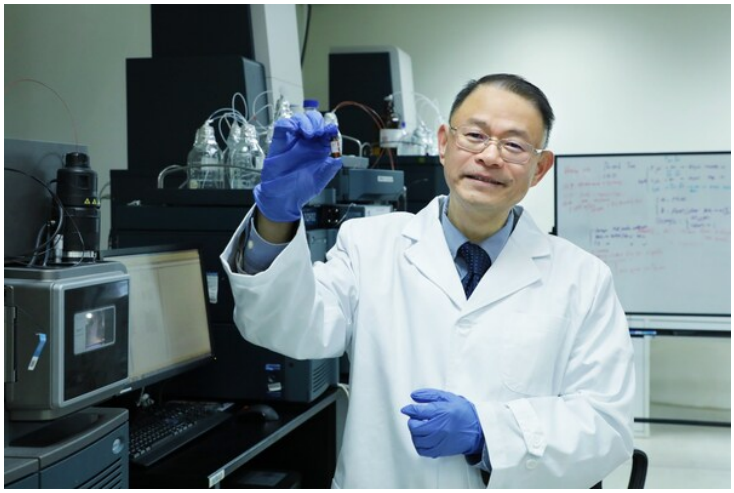

A research led by Hong Kong Baptist University (HKBU) and the Shanghai Sixth People's Hospital Affiliated to School of Medicine at Shanghai Jiao Tong University (Shanghai Sixth People's Hospital) has discovered that an aptamer developed by HKBU can be used to treat X-linked hypophosphatemia (XLH), a rare bone disease.

The aptamer, originally developed to treat osteogenesis imperfecta, has been granted Orphan Drug Designation and Pediatric Rare Disease Designation by the US Food and Drug Administration (FDA).

XLH ("X" denotes X-chromosome, a sex-determining chromosome) is a rare bone disease characterised by hypophosphatemia (i.e., low phosphate in blood). It is caused by a mutation in the *phosphate regulating endopeptidase homolog X-linked* (*PHEX*) gene, and its inheritance pattern follows an X-linked dominant mode. The bone tissue of XLH patients cannot mineralise properly, thereby affecting the hardness of the bones. The cartilage between the ribs of children with XLH grows and connects like beads strung together. They also result in limb deformities and growth retardation. Adult patients experience symptoms such as osteomalacia, bone pain, changes in body shape, shorter stature and pseudo-fractures, leading to reduced mobility or even disability.

According to the regulations of the US FDA, once a drug under development obtains Orphan Drug Designation, its subsequent research and development may allow for a reduction in the number of clinical trial samples, be exempt from new drug marketing authorisation fees, and enjoy seven years of market exclusivity. Pediatric Rare Disease Designation allows drugs to be reviewed with priority. Having been designated as both an orphan drug and a pediatric rare disease by the US FDA, Apc001 can accelerate its clinical translation and is expected to benefit patients with XLH sooner.

Currently, the pilot scale production of Apc001 has been completed and is undergoing the preclinical toxicological assessment by a third party. Apc001 is scheduled to enter clinical trials in both Mainland China and the US.