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ASIA EDITION

Perlara partners with Vivan Therapeutics to tackle rare genetic diseases

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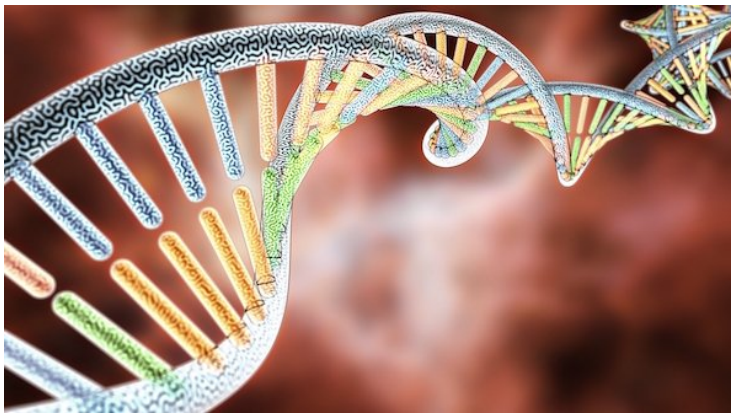

Perlara PBC, a rare disease public benefit company announced a rare genetic diseases collaboration with Vivan Therapeutics to identify novel therapeutics for rare genetic diseases.

Under this agreement, Perlara will refer highly motivated patients, families and foundations seeking to embark on a discovery journey for a rare genetic disease to Vivan Therapeutics. Vivan's technology known as the Personal Discovery Process (PDP), enables the design and generation of personalised fruit fly (*Drosophila melanogaster*) 'avatars'. Using robotics, thousands of FDA approved drugs and other drug libraries are screened in combinations to identify potential therapeutics.

Dr Nahuel Villegas, Chief Scientific Officer, Vivan Therapeutics comments, "Our fruit fly *in vivo* models are specifically designed to identify novel and unlikely therapies for diseases with clear genetic components. In this exciting collaboration with Perlara we will employ our unique platform to run massive drug screenings to define therapeutic options for diseases with no current standard of care treatments."

The first two diseases to be modelled are MAN1B1-CDG (congenital disorder of glycosylation) and ECHS1 deficiency. The ECHS1 gene and MAN1B1 gene are well conserved in fruit flies.

Individuals with MAN1B1-CDG have two deficient copies of the MAN1B1 gene. Currently, there are no treatments for this disease. Individuals with MAN1B1-CDG typically develop signs and symptoms of the condition during infancy, including a broad array of phenotypic abnormalities such as developmental disability, decreased muscle tone (hypotonia), delayed motor skills, and behavioural problems among others.

ECHS1 deficiency is a rare congenital metabolic disorder caused by biallelic mutations in the ECHS1 gene. Individuals with this mitochondrial condition typically have signs and symptoms of developmental delay, dystonia, seizures, and brain abnormalities.

Ethan Perlstein, CEO, Perlara commented, "We're excited to be working with Vivan on developing fly avatars of monogenic inborn errors of metabolism for rapid turnaround personalized drug repurposing. Flies are at the sweet spot of disease model complexity and clinical translatability."

Laura Towart, CEO, Vivan Therapeutics added, "This collaboration is our first outside of oncology and will enable us to utilise our platform technology to potentially identify therapeutics for diseases for which there are no treatments. We are looking forward to embarking on this important mission with Perlara and the families."