Tata Institute of Fundamental Research
Survey No. 36/P, Gopanpally Village, Serilingampally, Ranga Reddy Dist., Hyderabad – 500 046

Colloquium

Modulating SLC2A1 gene expression to treat brain energy failure

Umrao Monani

Columbia University Medical Centre, NY

The Monani lab at the Columbia University Medical Centre is engaged in the study of paediatric neurological diseases with a view to gaining a better appreciation of the selective vulnerability of neurons to death and dysfunction owing to perturbations in housekeeping proteins. One of these diseases, Glucose Transporter-1 deficiency syndrome (Glut1DS), is an especially useful paradigm and a major focus of the laboratory. Glut1DS is a prototypical brain energy failure syndrome with an urgent unmet medical need. It is caused by a haploinsufficiency of the SLC2A1 gene and, consequently, reduced levels of its translated product, the Glut1 protein. The disease is currently treated with high-fat diets, but these do not address its root cause – low Glut1 – and are modest in their effects. Raising Glut1 from the remaining intact SLC2A1 allele, which is present in most patients, is an intuitively appealing alternative therapeutic strategy. Dr. Monani's presentation will focus on one innovative means of doing so. Following from the identification of a novel natural antisense transcript that was found to concordantly regulate Glut1 expression, work in the Monani lab has shown how the transcript might be exploited to raise Glut1 activity and treat Glut1DS. Discussions of the unique Glut1DS phenotype, the therapeutic effects of the NAT in a mouse model of the disease and the challenges associated with translating pre-clinical findings into a safe, effective and viable clinical therapy will animate the presentation.

Monday, Jan 29th 2024

16:00 Hrs (Tea / Coffee 15:45 Hrs)

Auditorium, TIFR-H