Molecular diagnosis and therapeutic studies of spinal muscular atrophy (SMA) and duchenne muscular dystrophy (DMD); Challenges and opportunities in Malaysia

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Single-gene neuromuscular disorders, such as SMA and DMD, are among disorders with the greatest devastation to life. In many parts of the world, including Malaysia, we see tremendous magnitudes of the medical and social devastation they have imposed to the patients. No effective cures have been found, while molecular diagnoses sometimes hampered by complicated genomic conditions. Scientists from developing nations have actually been blessed by the abundance of diverse biological resources, especially those of plants, which reserves active compounds with possible molecular effects. These may provide some answers to the ongoing search for the cures of some of these disorders. Unique pathogenesis of SMA that involves inter-relation between the twin genes, SMN1 and SMN2, has imposed researchers with tremendous opportunity for curing the disorder. Different strategies in manipulating the gene for increasing the impaired expression of SMN protein have been proposed during the last 2 decades. Pharmacologic approaches are among those with the most promising. These approaches have ignited research interests on the possibility of exploring the ability of indigenous compounds on their potentials in lessenig the disease severity. DMD/BMD with its out-of-frame/in-frame characteristics of molecular pathogenesis has also inspired researchers in finding treatment for the disorder. Recent study on compound which skips specific exon for restoring the disrupted reading frame in Dystrophin have alert possible similar actions exerted by indigenous compounds.

Biography
Dr Teguh Haryo Sasongko is currently a Senior Lecturer at Human Genome Center, School of Medical Sciences, Universiti Sains Malaysia. He completed his training as a medical doctor at the School of Medicine, Universitas Gadjah Mada, Indonesia in 2003. Upon awarded the Japanese Government’s Monbukagakusho Fellowship, he pursued a doctoral training in the Graduate School of Medicine, Kobe University, Japan from 2003 – 2008. From 2008 onwards he started his service to Universiti Sains Malaysia. His main expertise is Human Molecular Genetics, with specialty on splicing modulation factors. His current research has been focusing on therapeutic approaches to manipulate splicing of exons for restoring defective reading frame in Duchenne Muscular Dystrophy as well as to restore defective splicing in Spinal Muscular Atrophy. In pursuing his research objectives he is trying to restore defective splicing by locally manipulating affected target codon as well as by chemical substance that works on targeted splicing factors. He has been instrumental in initiating a Malaysian research community which embarks on the therapy of genetic disorders; an effort concerted by multidisciplinary scientists from prominent Malaysian universities. Dr. Teguh has published many articles in national and international peer-reviewed journals and book chapters in the fields of Human Molecular Genetics. He is currently also serving as an editorial board member and reviewer for some international journals.

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