NEWS IN BRIEF

Gene Therapy for the Limb-Girdle Muscular Dystrophy

Dysferlinopathy caused by recessive pathogenic variation in the *dysferlin* gene (DYSF) is designated as Limb-Girdle muscular dystrophy 2B (LGMD2B) or LGMD R2 dysferlin-related. Defective *dysferlin* gene (DYSF) leads to lack of sarcolemmal protein dysferlin, which impairs sarcolemmal muscle repair by reducing secretion of the enzyme acid sphingomyelinase (ASM), ultimately leading to muscle degeneration. Gene therapy is one of the treatment modalities which acts on muscles by, making them capable of producing the missing proteins. But in case of LGMD2B, the large size of the mutated gene and body wide delivery to reach all muscles in the body are the significant challenges in front of researchers. Recently a team of researchers from Center for Genetic Medicine Research at Children's National Research Institute (CNRI), Washington developed a liver specific Adeno-associated virus (AAV) vector with a proposed mechanism that a single *in vivo* dose of an AAV vector produces a secreted version of human acid sphingomyelinase (hASM) in the liver, which then reaches muscles through blood and restores the membrane repair capacity of patient cells to healthy levels. The team demonstrated that hASM-AAV treatment restored myofiber repair capacity, attenuated fibro-fatty muscle degeneration, increased myofiber size, and restored muscle strength in LGMD2B mouse model. Translation of this research into clinical therapy in near future will be of much help to the patients of LGMD 2B.
(*Journal of Clinical Investigation 07 January, 2022*)

New way to deliver mRNA Vaccines

Needles – Not only children even some adults are also afraid of needle prick. This fear of needle results in low acceptance and adherence with injectable drugs and vaccines. A team of researchers from the Massachusetts institute of technology (MIT) have developed a capsule to deliver injectable drugs directly in the mucosal layer of stomach. In the initial two years, during trials the team had been successful in delivering large molecules like monoclonal antibodies into the stomach mucosa without degradation. Recently they have successfully delivered 150 micrograms of RNA in the stomach of pigs without degradation, which is more than the mRNA present in the COVID-19 vaccines in use at present. Subsequently, the research team documented a successful production of reporter protein by stomach cells. The researchers are now planning to create a systemic immune response in humans, including activation of B- and T-cells, by delivering mRNA vaccines using their capsule. The success of this modality might abolish the need of injection for the delivery of vaccines/drugs in near future, thus helping us to reach our vaccination targets.
(*Matter 31 January, 2022*)

Nano- and Micro-plastics: Danger to our future generations

Recently there are articles on 'Plasticenta' i.e., presence of microplastic particles in human placenta. Plastic, a synthetic material made of polymers, become an integral part of our present day lifestyle due to its features like light weight, durability, chemical and thermal resistance, and low cost. Globally, environmentalists are worried about the impact of plastic and its degradation products on our environment. But what about its effect on our own health? Nano- and micro-plastic particles are <1000 ìm and <1000 nm in size respectively, inside human body these particles can cross through the biological barriers, cause inflammatory reactions in sensitive areas and acts as carriers of various toxic chemicals causing long term effects on human health. As pregnancy, infancy, and childhood are sensitive periods of vulnerability to environmental toxicants exposures. In a recent paper from the Norwegian University of Science and Technology, authors studied the effects of exposure to nano- and microplastics (NMPs) through placental transfer, breastmilk, ingestion, inhalation, and dermal absorption on the health of the newborns and children. Their findings showed that there is a lack of substantial data regarding the effect of NMPs on the health of infants and children, yet they have given recommendations for the policymakers, industry as well as for the families. The important ones includes the appropriate measures to reduce the exposure of the children to NMPs – through steps like removal of the youth involved in plastic waste collection and e-waste burning, reducing the contact of plastic with food items intended for children, regular wet-cleaning of our homes, and careful choice of safer personal care products and building materials etc.
(*Environmental Health Perspectives 26 January, 2022*)

Vaccine against Zika Virus

Since 2015-16, when an outbreak was reported from Brazil, American and African region no treatment is available for Zika virus or its associated disease. It was declared as a public health emergency of international concern by WHO along with the guidelines to prevent its spread. But now a ray of hope has emerged in the form of Zika virus vaccine, which is the result of the collaboration between the Texas Biomedical Research Institute's Southwest National Primate Research Center (SNPRC), Trudeau Institute and Walter Reed Army Institute of Research (WRAIR), where the vaccine is under development. The initial test results of the purified, inactivated Zika vaccine (ZPIV) candidate in non-pregnant animal models, showed its effectiveness in clearing the virus from their blood. While on testing in pregnant mice and marmosets, the vaccine prevented fetal malformations and generated neutralizing antibodies in 80% and 90% cases, respectively. In Phase 1 human trials, it has shown to be safe and able to elicit a protective immune response. Though its, quite early but the results have shown that ZPIV is both potent and durable, and it also has the potential to prevent the harmful consequence of Zika virus infection during pregnancy.
(*npj Vaccines 27 January, 2022*)

RAJESH KUMAR MEENA
raj.mamc@gmail.com