



## Call For Scientific R & D Proposals

World Without GNE-Myopathy (India)

Centre for Drug Development

GNE myopathy is a progressive degenerative skeletal muscle disorder that causes extreme disability. It is an adult onset autosomal recessive disorder caused by biallelic mutations in GNE gene which is the rate limiting enzyme in the sialic acid biosynthetic pathway (see Nishino *et al.*, 2015 for review). Currently there is no therapy available for this disease. WWGM has decided to fund strategic research in gap areas in order to hasten the process of finding a therapy/cure for GNE myopathy. The funding is planned as seed funding for one year in order to help establish proof of principle. The budget limit for each project is rupees eight lakhs. Currently the proposals are being sought in the following areas.

1. Structural, biochemical and functional characterization of mutant GNE proteins (containing mutations seen in patients). The aim is to understand how mutations spread all across the gene are changing the protein to result in the common disease phenotype of inclusion body and rimmed vacuoles.
2. Expression of GNE gene in muscle cells and tissues using viral and non-viral vectors. The aim is to have a system that can deliver and express wild type GNE gene in the muscles of mice. Preference will be given if the vector has been shown to be non-toxic in human or animal models.

**Eligibility:** Applications may be submitted by researchers from both public and private organizations.

**How to apply and Assessment criteria:** Applicants should submit two hard copies and a soft copy to **Dr. A. Bhattacharya, Managing Trustee, WWGM**, 1123 Sector B1, Vasant Kunj, New Delhi – 1100070, Email: [wwgm.india@gmail.com](mailto:wwgm.india@gmail.com). The Format and terms and conditions are available at [www.gne-myopathy.org/](http://www.gne-myopathy.org/). The last date for submission is **December 31<sup>st</sup>, 2018**.

The proposals will be evaluated by the Scientific Advisory Committee of WWGM within eight weeks after the last date of submission and the successful applicants will be informed thereafter.

### Reference:

Nishino I, Carrillo-Carrasco N, Argov Z. GNE myopathy: current update and future therapy. *J Neurol Neurosurg Psychiatry*. 2015; 86(4):385–92. doi: 10.1136/jnnp-2013-307051 PMID: 25002140