

## Successfully accomplished/participated events

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### **World Orphan Drug Congress Asia (3-4 June) and the 1st meeting of the Asia Pacific Alliance of Rare Disease Organizations-APARDO (5 June 2015), Singapore**

Mr. M. Louis, Executive Director of Muscular Dystrophy Foundation India has been invited as a guest to both the events. The meeting of the APARDO will cover a variety of informative topics including: 'Orphan Drug Approvals and Access', 'Biopharmaceutical Innovation And Clinical Trials For Rare Diseases' and 'Health Communications'. The afternoon session will be an internal meeting to ratify its Constitution and plan next steps for the Alliance.

The World Orphan Drug Congress builds bridges amongst the orphan drug communities in Asia between Pharma companies, patients and regulators.

**World Orphan Drug Congress Asia** is the one stop shop for government, regulators, physicians, patient groups and industry to come together to develop sustainable market access strategies and overcome regulatory hurdles in Asia.

Showcasing successful case studies of orphan drug development in advanced nations like Japan, Korea, Taiwan and Australia, this event is dedicated to fostering partnerships and collaborations amongst the orphan drug community.

Jam packed across two days with numerous keynote presentations, interactive roundtable discussions and plenty of networking opportunities, you now can maximise your lead generation activities and meet with the people that matters most to Asia's orphan drugs market.

### **International workshop on, "Patient Organization Activism around the world" held at Brighton, United Kingdom during 18-19 May, 2015**

Mr.M.Louis, Executive Director of Muscular Dystrophy Foundation India has participated as a delegate in the workshop that was organized by the Centre for Bio networking, School of Global studies, Department of Anthropology, University of Sussex. Mr.Louis has also met with with many international organizations including Action Duchene, Parent Project Italy, Euro Stem Cells etc and visited few other organizations during his week long stay.

### **Open dialogue with M/s. SAREPTA Therapeutics, held on 06.04.2015**

You might be aware that Sarepta Therapeutics is one of the leading drug development companies engaged in developing 'Eteplirsen' – an exon skipping drug for Duchene Muscular Dystrophy patients.

In order to update the progress in seeking the approval of FDA for ETEPLIRSEN and discuss further in this regard with the patient support groups from across the world, Sarepta Therapeutics has organized a closed conference call on **06.04.2015 at 11.30 PM** (2.00 PM EDT).

M.Louis, Executive Director of Muscular Dystrophy Foundation India was invited by Dr. Edward M. Kaye, MD, the new CEO (interim), Sarepta Therapeutics to participate in the conference. Mr. M. Louis has participated and assured his fullest support in finding a cure for DMD.

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### **TREAT-NMD webinar on Post-Marketing Surveillance held on 21.01.2015**

**Mr. M. Louis, Executive Director of Muscular Dystrophy Foundation India has participated as an invitee and raised many pertinent questions including the scope of 'Translarna' and its global availability.**

The TREAT-NMD Executive Committee has recently consulted with representatives of all key stakeholders in the neuromuscular community to ascertain the feasibility of developing a disease-specific post marketing surveillance platform.

Post marketing surveillance (PMS) is part of the drug development process (also known as Phase IV) and is mandated by the pharmaceutical regulators (FDA, EMA and other national bodies). This is to allow for the collection of safety and efficacy data once a drug has received (conditional) marketing approval and to assess how it performs in 'the clinical setting'. PMS data has to be collected on every patient receiving the drug, or an agreed number of these patients, and is usually part of long-term data collection that can run for a period of about 10-15 years. For further details please visit <http://www.treat-nmd.eu/resources/post-marketing-surveillance/introduction/>

### **Building a strong voice for patients in India: Advocacy Network Founding Meeting-Habitat Centre, New Delhi, 12.11.2014.**

Jointly organized by Consumer Connexion, Association of Community Pharmacists of India and Daksham A Foundation. Mr. M.Louis, Executive Director of MDF INDIA has deliberated in this meeting that lead to the formation of a 'Patient's safety advocacy network' in India.

### **Roundtable on 'Patient needs and stem cell research in India' -Delhi, 2nd July 2014**

Organized by Centre for Bionetworking in Asia, University of Sussex in partnership with Research and Information System for Developing Countries (RIS), Delhi and the Department of East Asian Studies at Delhi University.

Mr. M. Louis, Executive Director of MDF INDIA has been invited to be one of the panelists of this round table conference. Patients/parents residing in and around Delhi are most welcome to participate and benefit from the conference.

About the Conference: Patients of terminal and incurable conditions have diverse medical and non-medical needs. These range from everyday requirements around care-giving, quality healthcare and emotional support to more specialized needs such as genetic testing, disabled-friendly buildings and access to information about treatment options. In addition to fulfilling these everyday needs, many people living with terminal or incurable conditions are anxious for a breakthrough in biomedical research which will lead to a treatment to improve their quality of life. For many conditions, stem cell therapy is seen to have significant potential and, despite the fact that it remains unproven, is actively sought after by many patients.

In this roundtable, panellists will be asked to address the importance of stem cell research in the specific context of Indian patients living with terminal conditions such as muscular dystrophy, spinal

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cord injury, diabetes and cardiovascular disease. At the moment, basic research is being conducted across the country in government-funded biomedical and scientific institutes as well as in many private sector settings. Significant clinical research is also taking place in India—however, this is largely conducted by international pharmaceutical companies for drugs which are not necessarily aimed at the Indian population. Panellists will be asked to present their views on whether, given these factors, investing in stem cell research and experimental therapy should be considered an important priority by policymakers, health care providers and patient organisations when considering patient needs.

### **“Investiture Ceremony” of JEEVANA School, Madurai - 21.06.2014**

Ms. G.Renganayaki Chairperson and M. Louis, Executive Director of our organization have been invited to be the chief guests for the ceremony. It is a solemn function where the Headgirl, the Vice Headgirl, the Headboy, the Vice Headboy and the Prefects take oath and badges are pinned. The chief guests will also honor the students who had done extremely well in the Public Exams this year and the teachers who had guided them.

### **STEM 2013**

STEM 2013 is the perfect amalgam of Science and Business. Research and Clinical Trials in Stem cell areas are proving advantageous in finding cures to many diseases. The resulting opportunity provides a good platform for commercialization. Industry is keen on benefitting from the prospects of business as already indicated by the big focus on regenerative medicine in a number of Pharma and Biotech companies.

STEM 2013 is building a common platform for the Scientists, Industry professionals, the academics and the Student fraternity to come together at this conference and network with each other and share their knowledge and experience in this subject which will lead to future path in the growth of new therapeutic area for the benefit of mankind. STEM 2013 speakers include scientists, researchers, industry professionals from biotechnological companies, pharmaceutical companies, doctors and academic faculties, IPR and Investment/Fund Managers/Bankers.