Press release

The 2nd meeting of the series Days of Molecular Medicine « The Translational Science of Rare Diseases : From Rare to Care »

Paris (France), October 25, 2012 – Each year the American Association for the Advancement of Science, which publishes the prestigious journals Science and Science Translational Medicine, the Karolinska institute in Stockholm, the DMMGF foundation and the Fondation Ipsen organise a series of meetings entitled Days of Molecular Medicine. This year the Institute for Molecular Biotechnology (Vienna) was also involved in the partnership. The meetings are highly regarded in the field of translational medicine, which bridges the gap between fundamental research and medical application. The latest event took place from 8-10 October in Vienna, Austria. Several well known scientists, including the Nobel Prize Eric Kandel attented the meeting.

Entitled "The translational science of rare diseases: Rare for Care", the meeting focused on an original topic with far-reaching consequences: research into rare illnesses and how it can lead to discovering therapies, including for common illnesses. Rare illnesses are also known as orphan diseases because they affect few patients and can therefore be neglected. Yet, although each rare illness only affects a few patients, together, they affect a lot of people. Their treatment also plays an essential role in adapting our approach to human physiology and pathology. Many rare illnesses have genetic origins that can be elucidated. Pinpointing the gene or genes involved enables a protein or a biochemical path to be targeted and acted on, using gene therapy or drugs. Cystic fibrosis is a remarkable example of this. Many pathogenic mutations have been identified and their mechanism brought to light through research into the disease. As Peter Mueller (Cambridge, USA) demonstrated in Vienna, this resulted in a drug formulation that can increase a patient's life expectancy by twenty years!

Concerning gene therapy, Alain Fischer (Hôpital Necker, Paris, France) presented his pioneering work, while several others presented on-going trials to treat Canavan disease or blindness. Many extremely rare and newly identified illnesses were also presented, among them those discovered through a special programme instigated by the American National Institute of Health.

About the *Fondation Ipsen*

Established in 1983 under the aegis of the Fondation de France, the mission of the Fondation Ipsen is to contribute to the development and dissemination of scientific knowledge. The long-standing action of the Fondation Ipsen aims at fostering the interaction between researchers and clinical practitioners, which is indispensable due to the extreme specialisation of these professions. The ambition of the Fondation Ipsen is to initiate a reflection about the major scientific issues of the forthcoming years. It has developed an important international network of scientific experts who meet regularly at meetings known as Colloques Médecine et Recherche, dedicated to six main themes: Alzheimer's disease, neurosciences, longevity, endocrinology, the vascular system and cancer science. Moreover the Fondation Ipsen has started since 2007 several meetings in partnership with the Salk Institute, the Karolinska Institutet, the Massachusetts General Hospital, the Days of Molecular Medicine Global Foundation as well as with the science journals Nature, Cell and Science. The Fondation Ipsen produced several hundreds publications; more than 250 scientists and biomedical researchers have been awarded prizes and research grants.

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