Thalassemia and Sickle Cell Disease

Time for a New Optimism

Bilateral Workshop

UCSF Benioff Children's Hospital, Oakland, USA and
Postgraduate Institute of Medical Education & Research, Chandigarh, India

November 5-6, 2016

Chandigarh, India

Sponsor: Indo-US Science and Technology Forum

UCSF Benioff Children's Hospitals

POSTGRADUATE INSTITUTE OF MEDICAL EDUCATION AND RESEARCH CHANDIGARH

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Technical Agenda

DAY 1: November 5, 2016
Standards of Care for Hemoglobin Disorders

Keynote 1: The Challenges of Building an International Collaboration for Medical Research

Session I: Epidemiology of Hemoglobin Disorders

- ◊ SCD and Thalassemia – Changing patterns, emerging Issues, and research priorities
- ◊ Complex distribution of hemoglobin disorders in India – From a regional to national perspective
- ◊ Newborn screening for hemoglobin disorders – The California experience

Session II: Sickle Cell Disease

- ◊ Determinants of severity of SCD in India: What we know
- ◊ The Infant with SCD: Early perils and risk assessment
- ◊ Complications and crises: Early detection and prevention
- ◊ Adults with SCD: Keeping track
- ◊ SCD care in India: Out in the field

Session III: Clinical Issues in Thalassemia

- ◊ Management of the transfusion-dependent patient
- ◊ Thalassemia: Major or Intermedia?
- ◊ The burden of E-beta thalassemia in India
- ◊ Carrier screening for thalassemia

Session IV: Common Issues in Management

- ◊ Living with a chronic illness
- ◊ Transition of care – What the future holds
- ◊ Iron Chelation Therapy: Single or Combined
- ◊ Allo-immunization: An ounce of prevention

DAY 2: November 6, 2016
Translational Research Initiatives in Hemoglobin Disor-

Session V: Diagnosis of Hemoglobinopathies

- ◊ Screening for SCD and thalassemia in India
- ◊ Chorionic villus sampling for early prenatal diagnosis
- ◊ Nextgen sequencing for beta globin disorders: towards non-invasive prenatal diagnosis

Session VI: Advances in Treatment

- ◊ Repairing erythropoiesis: Hepcidin and Activin-inhibitors
- ◊ Therapeutic Targets for SCD
- ◊ Hydroxyurea: Efficacy studies and access in India

Session VII: Stem Cell Transplantation

- ◊ Stem cell transplantation in thalassemia: Standard and experimental approaches
- ◊ Stem cell transplantation – the Vellore experience
- ◊ SCT for SCD: when and how

Keynote 2: Role of philanthropy in targeted advanced medical training: multiplier effect?

IUSSTF: Building bridges between scientific communities

Session VIII: Gene therapy

- ◊ The development of gene therapy for hemoglobin disorders
- ◊ CRISPR/Cas9: the promise of gene editing
- ◊ Clinical trials of gene therapy for beta thalassemia
- ◊ Panel Discussion: Bringing Gene Therapy into Clinical Practice

Symposium Closing:
Regulatory Environment, Challenges and Opportunities for Collaboration

The ***goal*** of this symposium is to develop collaborations between clinicians and scientists from the United States and India, which will serve to accelerate the development of breakthrough therapies for thalassemia and sickle cell disease. India has the largest population in the world affected with these serious inherited blood disorders. The majority of patients become sick soon after birth and many die during childhood due to inadequate treatment. The resources required to provide care - blood transfusions and expensive drugs - are an enormous burden on the health system.

In the US, sickle cell disease is a public health and national research priority for which universal newborn screening has been implemented. The prevalence of beta thalassemia, a minority disease, is rising due to immigration.

Several recent critical scientific advances in prenatal diagnosis, iron metabolism, erythropoiesis and gene therapy have the potential to revolutionize the entire field. There is a renewed hope that the suffering of individuals living with thalassemia and sickle cell disease can be reduced. Fast-track development of these discoveries is an imperative that can be realized through a reciprocal and cooperative scientific relationship between academic medical centers in India and the United States.

On the front page: The Open Hand (La Main Ouverte), Chandigarh