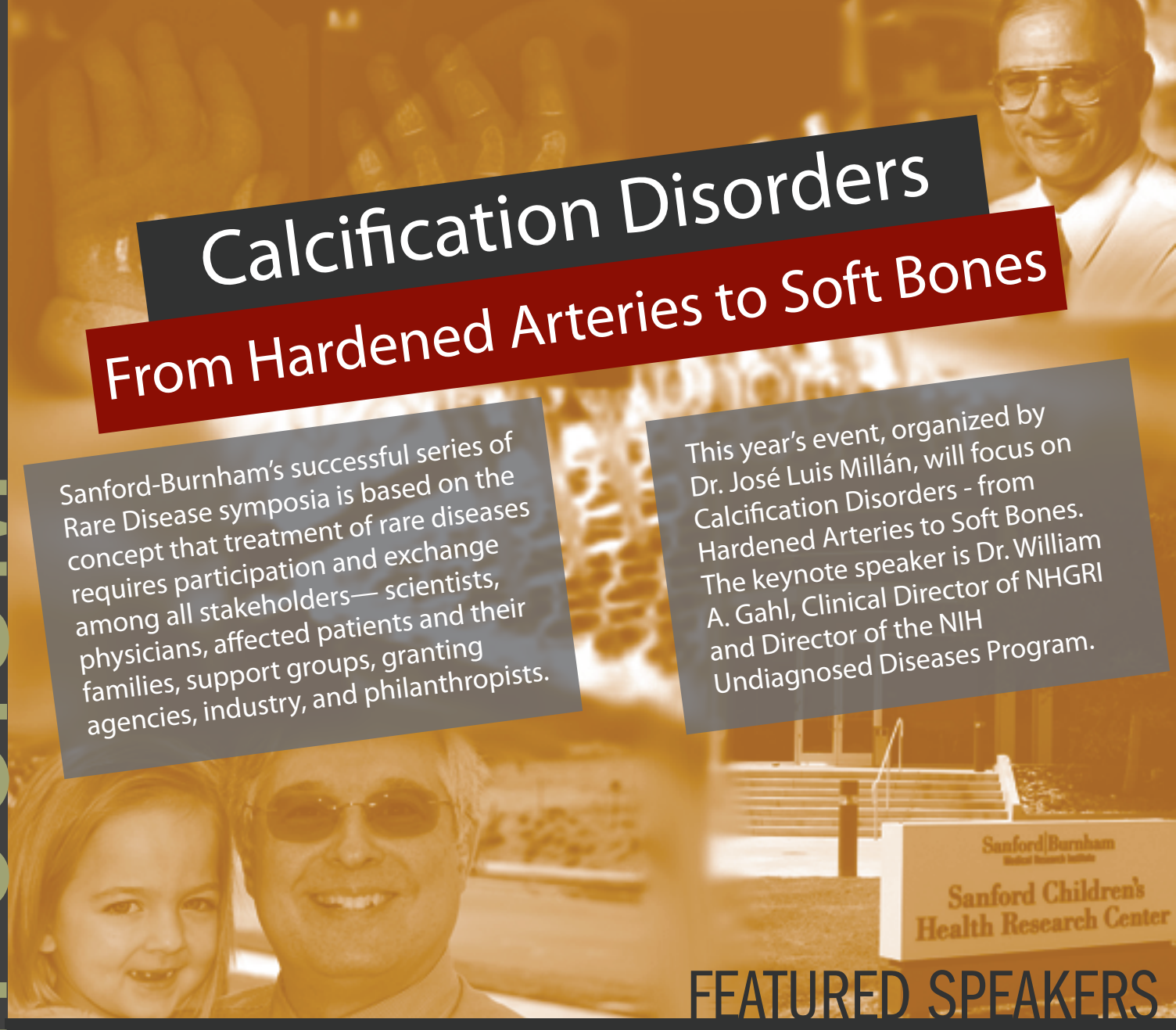
4th ANNUAL 2013

Sanford-Burnham RARE DISEASE DAY SYMPOSIUM



Calcification Disorders
From Hardened Arteries to Soft Bones

Sanford-Burnham's successful series of Rare Disease symposia is based on the concept that treatment of rare diseases requires participation and exchange among all stakeholders— scientists, physicians, affected patients and their families, support groups, granting agencies, industry, and philanthropists.

This year's event, organized by Dr. José Luis Millán, will focus on Calcification Disorders - from Hardened Arteries to Soft Bones. The keynote speaker is Dr. William A. Gahl, Clinical Director of NHGRI and Director of the NIH Undiagnosed Diseases Program.

February 28, 2013
8:30am-5:30pm

10905 Road to the Cure
La Jolla, CA

free registration online
sanfordburnham.org

FEATURED SPEAKERS

8:45-11:30 - Session 1: Diagnosis and Treatment of Vascular Calcification

KEYNOTE ADDRESS
The NIH Undiagnosed Diseases Program: New insights using genomic methodologies
William A. Gahl, M.D., Ph.D.
Clinical Director, NHGRI
Director, NIH Undiagnosed Diseases Program

New insights into the development of aortic calcification and MGP deficiency
Hervé Kempf, Ph.D.
Researcher, Université de Lorraine, France

Generalized arterial calcification of infancy and pseudoxanthoma elasticum: Two faces of the same coin
Frank Rutsch, M.D.
Professor, Muenster University Children's Hospital, Germany

Phosphatase inhibitors for the prevention and treatment of medial vascular calcification
José Luis Millán, Ph.D.
Professor, Sanford-Burnham Medical Research Institute

11:30-12:30 - Lunch

12:30-2:30 - Session 2: Pathophysiology and Management of Soft Bones

Update on bone-targeted enzyme replacement therapy for hypophosphatasia
Michael P. Whyte, M.D.
Medical-Scientific Director, Center for Metabolic Bone Disease and Molecular Research, Shriners Hospital for Children

Recessively inherited osteogenesis imperfecta and the biology and medicine of defects in collagen processing
Peter H. Byers, M.D.
Professor, Departments of Pathology and Medicine (Medical Genetics), University of Washington

FGF23: a common denominator in metabolic bone diseases
Kenneth E. White, Ph.D.
Associate Professor of Medical and Molecular Genetics, Indiana University

X-linked hypophosphatemia 2013: a clinical update of the prototype renal phosphate wasting disorder
Thomas O. Carpenter, M.D.
Professor of Pediatrics (Endocrinology) and of Orthopaedics and Rehabilitation and Clinical Professor of Nursing
Director, Yale Center for X-Linked Hypophosphatemia
Medical Director, Hospital Research Unitrch

2:45-5:30 - Session 3: Pathophysiology and Management of Soft-Tissue Ossification

Heterotopic bone formation in fibrodysplasia ossificans progressiva (FOP) and progressive osseous heteroplasia (POH)
Eileen M. Shore, Ph.D.
Cali and Weldon Research Professor, University of Philadelphia

MHE: Pathogenic mechanisms and prospects for therapies
Maurizio Pacifici, Ph.D.
Director of Research, Division of Orthopaedic Surgery, The Children's Hospital of Philadelphia

Genetic skeletal diseases caused by non-heritable mutations, such as Gorham-Stout and CLOVES
Matthew L. Warman, M.D.
Director, Orthopedic Research Laboratories, Boston Children's Hospital

4:15-5:15
Panel Discussion: Patient Advocacy Groups
Charlene Waldman Session Chair

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ORGANIZER
José Luis Millán, Ph.D.
Professor, Genetic Disease Program, Sanford Children's Health Research Center