



**1st International Gaucher Meeting
on Bone Disease**

Rio de Janeiro, July 5th-6th, 2013
Hotel Windsor Barra
Scientific Program

Chair

Pramod Mistry (USA)

Hosts

Ana Maria Martins (Brazil)
Jose Simon (Brazil)



July 5th to 6th, 2013
Rio de Janeiro, Brazil

Scientific Program



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Day 1 - Friday, July 5th

09:00 - 09:20	Welcome <i>Rogério Vivaldi (USA)</i>
09:20 - 09:30	Introduction <i>Pramod Mistry (USA)</i>
09:30 - 10:15	Gaucher disease: a new era of delineation and therapy <i>Pramod Mistry (USA)</i>
Plenary Session 1	Gaucher bone disease: a review <i>Pramod Mistry (USA)</i>
10:15 - 10:45	Systemwide effects of GBA1 deficiency <i>Mone Zaidi (USA)</i>
10:45 - 11:15	Coffee break
11:15 - 11:45	Biochemical markers of bone turnover in Gaucher disease <i>Cynthia Brandão (Brazil)</i>
11:45 - 12:15	Radiological assessments of bone disease in Gaucher disease <i>Alberto Vieira (Portugal)</i>
12:15 - 12:30	Discussion <i>All</i>
Plenary Session 2	Management of Gaucher bone disease in adults <i>Ravi Kamath (USA)</i>
12:30 - 13:00	Patient-centered outcomes with imiglucerase: a transformational therapy <i>Pramod Mistry (USA)</i>
13:00 - 13:30	Long-term outcomes in adults: focus on bone <i>Ana Maria Martins (Brazil)</i>
13:30 - 14:30	Lunch

Plenary Session 3	Management of Gaucher bone disease in children <i>Ana Maria Martins (Brazil)</i>
14:30 - 15:00	ERT and therapeutic goals in pediatric population <i>Aneel Khan (Canada)</i>
15:00 - 15:30	Long-term Gaucher Registry data in pediatric population <i>Jose Simon (Brazil)</i>
15:30 - 15:45	Discussion <i>All</i>
15:45 - 16:00	Coffee break

Workshops

Workshop 1 - Imaging

Ravi Kamath (USA)

16:00 - 17:00
Session 1

17:00 - 18:00
Session 2

Workshop 2 - Clinical assessment on bone disease

Guillermo Drelichman (Argentina)

16:00 - 17:00
Session 1

17:00 - 18:00
Session 2

Workshop 3 - Quantification of bone material

Roger Zebaze (Australia)

16:00 - 17:00
Session 1

17:00 - 18:00
Session 2

Please, choose two subjects. One for each session

20:00 - 22:00

Group dinner

Day 2 - Saturday, July 6th

9:00 - 10:00	Workshops highlights
Plenary Session 4	Bone disease - predictors and assessments <i>Mone Zaidi (USA)</i>
10:00 - 10:30	Response to ERT using BMB score <i>Patricia Robertson (Australia)</i>
10:30 - 11:00	Natural history of Gaucher disease <i>Pramod Mistry (USA)</i>
11:00 - 11:30	Substrate reduction therapy and bone disease <i>Martha Dragosky (Argentina)</i>
11:30 - 12:00	Coffee Break
Plenary Session 5	Gaucher disease: history and future <i>Guillermo Drelichman (Argentina)</i>
12:00 - 13:00	Gaucher disease as a case study in the approach to drug development <i>Seng Cheng (USA)</i>
13:00 - 14:00	Lunch
14:00	Departures

