

**1<sup>st</sup> European  
Gaucher Leadership Forum  
“Building on Today’s Standard of Care”**

**14<sup>th</sup>-15<sup>th</sup> May, 2009  
University of Milan  
Milan, Italy**

**Chair**

Prof. M.D. Cappellini (*Italy*)

**Scientific Steering Committee**

Dr. N. Belmatoug (*France*)

Prof. T.M. Cox (*United Kingdom*)

Prof. Dr. med. Stephan vom Dahl (*Germany*)

Prof. A. Zimran (*Israel*)

**Thursday, May 14<sup>th</sup>, 2009**

Timings	Topic	Chair(s)	Speaker
Morning	Arrivals		
12:30-13:30	Lunch and poster viewing		
13:30-13:45	Start of Meeting		
	Welcome and introduction to the 1 <sup>st</sup> European Gaucher Leadership Forum and to the University of Milano	Prof. M.D. Cappellini	
<b>S-1</b>	<b>Session One</b> Haematological aspects of Gaucher Disease	Prof. M.D. Cappellini / Dr. P.B. Deegan	
13:45-14:05	Focus on haematological abnormalities and pathophysiological mechanisms		Prof. A. Zimran
14:05-14:25	Haematological algorithms in Gaucher disease: Outcomes from an international meeting		Prof. P.K. Mistry
14:25-14:45	<u>Case Study</u> Classical haematological abnormalities		Dr. S. Linari

Thursday, May 14<sup>th</sup> (continued)

Timings	Topic	Chair(s)	Speaker
14:45-15:05	Dried blood spot test for screening: Update on high output screening process with new substrate		Dr. J. Keutzer
15:05-15:25	Molecular analyses in pursuit of diagnosis		Dr. M. Filocamo
15:25-15:45	Discussion S-1		
15:45-16:10	Break and poster viewing ( <i>interactive format for poster session</i> )		
<b>S-2</b>	<b>Session Two</b> Chronic inflammations and immune response	Prof. T.M. Cox/ Dr. N. Belmatoug	
16:10-16:40	The biochemistry of lipid storage and inflammatory mechanisms		Prof. K. Sandhoff
16:40-17:00	<u>Case study</u> MGUS/Myeloma (haematological algorithms)		Prof. A. El-Beshlawy
17:00-17:20	General Q&A Day One Open and interactive discussion	Prof. M.D. Cappellini, Dr. N. Belmatoug, Prof. Cox	
17:20-17:30	Conclusion	Prof. T.M. Cox/ Dr. N. Belmatoug	
20:00	Meet in hotel lobby for transfer to dinner event		
20:30	Conference Dinner		

Friday, May 15<sup>th</sup>, 2009

Timings	Topic	Chair(s)	Speaker
09:00	Start of Day Two		
09:00-09:10	Welcome back and introduction to Day Two	Prof. M.D. Cappellini	
<b>S-3</b>	<b>Session Three</b> Changing the course of the disease	Prof. S. vom Dahl / Dr. H. Michelakakis	
09:10-09:30	Management of Gaucher Disease as a chronic disorder		Dr. D.A. Hughes
09:30-09:50	Can biomarkers help monitor? – Outcomes from an international meeting and research direction		Prof. T.M. Cox
09:50-10:10	Disease severity scoring systems Interactive session with case study and follow up		Dr. M. Di Rocco
10.10-10.30	Discussion S-3		
10:30-11:00	Break and poster viewing ( <i>interactive format for poster session</i> )		
<b>S-4</b>	<b>Session Four</b> Special considerations in bone disease	Prof. L.W. Poll / Dr. M. Maas	
11:00-11:20	Preventing the irreversible: Time to AVN, treatment intervention and bone management guidelines		Prof. S. vom Dahl
11:20-11:40	Bone disease scoring and management		Prof. G. Mariani
11:40-12:00	<u>Case Study</u> Interactive case study discussion on bone with follow up		Dr. E. Lebel
12.00-12.20	Discussion S-4		
12:20-13:30	Lunch and poster viewing ( <i>interactive format for poster session</i> )		

Friday, May 15<sup>th</sup> (continued)

Timings	Topic	Chair(s)	Speaker
<b>S-5</b>	<b>Session Five</b> "Hot topics" in Gaucher Disease	Prof. A. Zimran / Dr. A. Tylki-Szymanska	
13:30-14:00	ITP in Gaucher Disease		Dr. H. Rosenbaum
14:00-14:30	Considerations for pregnant Gaucher patients: Challenges for the patient and physician		Dr. H. Belmatoug
14:30-15:00	<u>Case conundrums</u> Audience is invited to submit and present patient case that they are finding difficult to manage to ask a panel of experts for their opinion	Panel on podium	
15:00-15:30	Coffee break		
<b>S-6</b>	<b>Session Six</b> Back to the future	Prof. M.D. Cappellini / Dr. P.K. Mistry	
15:30-15:50	Small molecule: Latest data and future direction		Prof. E. Lukina
15:50-16:10	<u>Satellite Presentation</u> Public policy priorities and challenges for rare diseases		Dr. E. Tambuyzer
16:10-16:25	Update from European Gaucher's Association		Mr. J. Manuel
16:25-16:40	Poster prize awards two award winning poster presentations one "Learning and Leadership" Award		Scientific Steering Committee
16:40- 17:25	Key note presentation		Prof. S. Karlsson
17:25-17:45	Closing remarks from Prof. Cappellini on behalf of Scientific Steering Committee  Gaucher Leadership Forum Evaluation Form		Prof. M.D. Cappellini
17:45	End of Day Two – Farewell coffee		