

# 7<sup>th</sup> Gaucher Leadership Forum- International Meeting

## *From Scientific Evidence to Patient Care*

Copenhagen, 31 March – 1 April 2017

### PROGRAM

<b>DAY 1 – FRIDAY 31 MARCH 2017</b>		
13:00–13:30	Welcome and Introduction	Prof Claus Niederau, Germany, GLF chair Prof Allan Lund, Denmark, GLF Host
<b>KEYNOTE SPEAKER</b> Chair: Prof Claus Niederau, Germany		
13:30-14:00	Key note lecture: Gene Editing	Prof Stefan Karlsson, Sweden
<b>PLENARY SESSION 1: TRANSLATIONAL SCIENCE</b> Chair: Prof Timothy Cox, UK		
14:00-14:30	Underlying immunology in Gaucher disease	Dr Derralynn Hughes, UK
14:30-15:00	Alternative substrates, lipids and other biomarkers	Prof Hans Aerts, Netherlands
15:30- 16:00	CNS inhibition	Dr Seng Cheng, US
<b>15.00 – 15.30 COFFEE BREAK</b>		
<b>PLENARY SESSION 2: DIAGNOSTIC CHALLENGES</b> Chair: Dr Hanna Rosenbaum, Israel		
16:00 - 16:30	Examining the gap between prevalence and diagnosis	Prof Pramod Mistry, US
16:30 - 17:00	Screening and diagnostic approaches	Prof David Kasper, Austria
17:00 – 17:30	Approaches in specific situations – the Italian experience	Prof Maria Domenica Cappellini, Italy
17:30 – 18:00	Parkinson's disease	Dr Ellen Sidransky, US
18.00	<b>CLOSING DAY 1</b>	Prof Claus Niederau, Germany

This meeting is sponsored by Sanofi Genzyme.  
All speakers at this meeting are expressing their personal opinion  
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### PROGRAM

<b>DAY 2 - SATURDAY 1 April 2017</b>		
<b>WORKING GROUPS (Session I and II Rotation)</b>		
<b>SESSION I</b> <b>08:30-10.00</b>	<b>Working Group 1: Neurological aspects in Gaucher disease</b>	<b>Chairs: Prof Timothy Cox, UK &amp; Prof Allan Lund, Denmark</b> Speakers: Prof Alessio Di Fonzo, Italy & Dr Pablo Sardi, US
	<b>Working Group 2: Malignancy In Gaucher disease</b>	<b>Chairs: Prof Neal Weinreb, US &amp; Dr Derralynn Hughes, UK</b> Prof Madhav Dhodapkar, US & Prof Stephan vom Dahl, Germany
	<b>Working Group 3: Optimal management of children with Gaucher disease</b>	<b>Chairs: Prof Paige Kaplan, Prof Anna Tylki-Szymańska</b> Speakers: Dr Eugen Mengel, Germany & Prof Magy Abdelwahab, Egypt
	<b>Working Group 4: Challenging clinical cases in Gaucher disease</b>	<b>Chairs: Dr Nadia Belmatoug, Prof Claus Niederau</b> Speaker: Prof Elena Lukina, Russia
<b>10.00 – 10:30 COFFEE BREAK</b>		
<b>SESSION II</b> <b>10:30-12:00</b>	<b>Repeating Session I Working groups</b>	
<b>12.00-13.00 LUNCH</b>		
<b>PLENARY SESSION 3: Substrate Reduction Therapy</b>		<b>Chair: Prof Hans Aerts, Netherlands</b>
<b>13:00-13:30</b>	<b>Real world experience</b>	<b>Prof Pramod Mistry, US</b>
<b>13:30-14:00</b>	<b>Long term experience</b>	<b>Prof Elena Lukina, Russia</b>
<b>14:00-14:30</b>	<b>Practical aspects of SRT</b>	<b>Prof Claus Niederau, Germany</b>
<b>14:30-15:00</b>	<b>SRT Treatment Guidelines (EU vs US)</b>	<b>Dr Nadia Belmatoug, France</b>
<b>15.00-15.30</b>	<b>Summary 7th GLF Meeting and Closing</b>	<b>Prof Claus Niederau, Germany &amp; Prof Allan Lund, Denmark</b>

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