

GAUCHER EXPERT SUMMIT 2017

16-17 June 2017, Warsaw, Poland

Chairs: Professor Atul Mehta, Professor Anna Tylki-Szymańska and Professor Ari Zimran



Gaucher disease: Patient care across the generations

DAY 1 Programme: Friday 16 June

| Time | Presentation | Faculty |
|--|---|-----------------------------|
| 08:30 | Welcome to Poland | Anna Tylki-Szymańska |
| 08:40 | Welcome from Shire | Hartmann Wellhoefer |
| 08:50 | Chairs' welcome | Chairs |
| Scientific Session I - Addressing Gaucher disease in the early years | | |
| Session Chairs: Gregory Pastores & Anna Tylki-Szymańska | | |
| 09:00 | Diagnosing Gaucher disease in paediatric patients | Anna Tylki-Szymańska |
| 09:30 | Clinical management of paediatric Gaucher disease | Gregory Pastores |
| 09:50 | What have we learnt from GOS in paediatrics and pregnancy? | Heather Lau |
| 10:10 | Panel Q&A | All |
| 10:40 | Coffee | |
| Scientific Session II - Biomarkers in future Gaucher disease management | | |
| Session Chairs: Maria Cappellini & Atul Mehta | | |
| 11:00 | Early diagnosis of Gaucher disease in adult patients | Atul Mehta |
| 11:20 | Monitoring biomarkers in Gaucher disease: Is Lyso-Gb1 more than just a diagnostic tool? | Jeff Szer |
| 11:50 | Is there a relationship between bone biomarkers and bone manifestations of Gaucher disease? | Derralynn Hughes |
| 12:10 | Panel Q&A | All |
| 12:35 | Lunch | |
| 13:35 | Keynote lecture - An overview of lysosomes and lysosomal diseases | Bryan Winchester |
| 14:15 | Poster session | |
| 15:15 | Coffee | |
| Scientific Session III - Clinical trial design: It's all in the detail | | |
| Session Chairs: Patrick Deegan & Ari Zimran | | |
| 15:30 | Challenging case studies: Audience briefing | Maria Cappellini and Chairs |
| 15:45 | Keynote lecture - The importance of clinical trial design | Mario Cozzolino |
| 16:15 | Overview of clinical trial designs in Gaucher disease | Deborah Elstein |
| 16:35 | Interpretation of clinical trials in Gaucher disease | Ari Zimran |
| 16:55 | Panel Q&A | All |
| 17:15 | Summary and closing remarks | Chairs |
| 17:20 | Close of Day 1 | |



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DAY 2 Programme: Saturday 17 June

| Time | Presentation | Faculty |
|---|---|--------------------------------|
| 08:30 | Chairs' welcome | Chairs |
| Scientific Session IV - Managing Gaucher disease over the long term Session Chairs: Derralynn Hughes & Elena Lukina | | |
| 08:35 | Molecular and immunogenic profiles of ERTs | Ari Zimran |
| 09:00 | Therapeutic management of Gaucher bone disease | Nadia Belmatoug |
| 09:25 | Long-term clinical experience with ERT and SRT in adults with Gaucher disease | Derralynn Hughes |
| 09:50 | What have we learnt from GOS? | Patrick Deegan |
| 10:10 | Panel Q&A | All |
| 10:40 | Coffee | |
| 11:00 | Challenging case studies: Expert panel discussion | Maria Cappellini and Chairs |
| 12:00 | Keynote lecture - Historical perspectives of Gaucher disease: From the past to the future | Gregory Grabowski |
| 12:40 | Closing remarks | Chairs |
| 12:50 | Lunch and departures | |