Drug Development for Rare Diseases: Where are we now, how did we get here and where do we want to be?
Lessons Learned from Treating Lysosomal Storage Disorders.

4-5 November 2021 Online
All times CET (Paris/Amsterdam)

Thursday 4 November

1400-1410 **Welcome**
Sandra Sirrs, Vancouver; Robin Lachmann, London

Session 1: ***Drug development for rare diseases***

1410-1430 **What makes a successful treatment? Clinical experience of treating LSDs.**
Robin Lachmann, London
Contrast ERT for Gaucher with other ERTs. Introduce reversibility versus 'stabilisation'. Introduce heterogeneity of patient populations and individual responses. Talk about different 'windows of opportunity for treatment" and value of biomarkers in this context

1430-1450 **Natural history, divergent phenotypes and how these impact clinical trial design – the example of spinal muscular atrophy and mitochondrial disease**
Mark Tarnpolsky, Hamilton

1450-1510 **Designing trials with small numbers of patients**
Robin Ristl, Vienna

1510-1530 **Discussion**

1530-1550 **Break**

Session 2: ***Treatment of LSD's: how have we arrived where we are now?***

1550-1610 **Pompe: a qualified success story**
Hannerieke van den Hout, Rotterdam

1610-1630 **Mucopolysaccharidoses: slowing progression and accumulating complications**
Uma Ramaswami, London

1630-1650 **Fabry: struggling to see the benefits in a diverse population**
Robin Lachmann, London

1650-1710 **Discussion**
1710-1730 **Break**

Session 3: ***Regulatory aspects of orphan drugs***

1730-1750 **Does Orphan Drug Legislation work?**
Aidan Hollis, Calgary
What is the legislation? Has it been successful in bringing more drugs to market? What are the most problematic parts of the legislation?

1750-1810 **Selecting meaningful endpoints: What constitutes efficacy?**
Sebastian Schneeweiss, Boston
How do we reconcile what pharma and regulators want to measure with what payers need to see in lifelong conditions.

1810-1830 **Do we need special procedures for the assessment of orphan drugs?**
Karen Facey, Edinburgh

1830-1850 **Discussion**

1850-1900 **Summing up**

End of Day 1

Friday 5 November

1400-1410 **Welcome**

Session 5: ***Pricing of orphan drugs***

1410-1430 **Orphan Drug Development: What does it cost?**
Kavisha Jayasundara, Toronto
Does it really cost more to develop orphan drugs?

1430-1450 **What is an acceptable price in rare genetic diseases?**
Mike Drummond, York
How do regulators decide about reimbursement of treatments that cost more than the agreed limit per QALY?

1450-1510 **Prioritisation and access for rare diseases in the developing world**
Juan F Cabello, Macul (Chile)

1510-1530 **Discussion**

1530-1550 **Break**

Session 6: ***What happens after authorization?***

1550-1610 **Post marketing initiatives: lessons from oncology**
Sheela Upadhyaya, London

1610-1630 **Post-marketing registries**
Sandra Sirrs, Vancouver
How do we get useful, unbiased and open post marketing surveillance?

1630-1650 **Break**

Session 7: ***Orphan drug authorisation and access: towards new models***

1650-1710 **Private meets public: how can we improve access for a reasonable price?**
Saco de Visser, The Hague

1710-1730 **Public and patient engagement**
Alastair Kent, London
a. what the literature shows on public perceptions around willingness to pay for rarity and b. how you could design a role for public engagement as part of ongoing consultation process for new drug approvals

1730-1750 **Adaptive pathway approach: incentives for pharmaceutical companies**
Wendy Olsder, Eindhoven

1750-1810 **Discussion**

1750-1800 **Close**