



DANSK BIOTEK

Biotek Salon#15

11 June 2018 from 14:00-17:00

COBIS, Ole Maaløes Vej 3, 2200 Copenhagen N

Rare Disease Workshop

DANSK BIOTEK & Syneos Health invite you to a Rare Disease Workshop

Agenda:

14:00 Registration and networking

14:30 Welcome

Anders Hinsby – Board Member, DANSK BIOTEK

14:40 Clinical Development Trends in Rare Disease

Presented by **Cinzia Dorigo** (Vice President Clinical Development – Rare Disease Consortium Lead)

- Market and regulatory trends in rare disease clinical development
- Recent experience with adaptive pathway submission process, and new umbrella protocol design
- Innovative model of collaborative approach for patient centric strategies
- Collaboration and experience with European Reference Networks to support rare disease clinical development

15:20 Integrated Real World Evidence to Support Rare Disease Clinical Development & Commercialization – presented by **Maryna Kolochavina** (Project Director, Real World & Late Phase)

- Discussion about the Real Word Evidence collection impact on clinical development design
- Early access demand and balance with safety and efficacy
- Strategies to manage the challenge of the data fragmentation
- Recommendation for the proper Real-World Evidence design

16:00 Market Access in Rare Disease – Considerations in Early Development – presented by **Doug Niven** PhD (Senior Engagement Manager, Pricing and Market Access) and **Monique Martin** (Managing Director, Consulting)

- Impact of orphan designation on market access – timelines/requirements.
- Use and importance of early HTA advice/guidance process in key markets (Nordic HTA approaches/processes)
- Implications of choice of comparators; endpoints; inclusion/exclusion criteria for market access and HTA
- The importance of early modeling/pricing to understand the pricing limits and reimbursement hurdles
- How market access considerations impact the 'valuation' of a product when selling or licensing it

16:40 Networking and refreshments

Register here:

The event is free of charge for members, however, non-members will be charged a fee of 500 DKK