Amicus, Wednesday August 31, 2022, 07.30-08.30, Hall Jerusalem

The FACEs* of Fabry disease
*FACEs: Fabry Associated Clinical Events – renal, cardiac and cerebrovascular outcomes

Welcome and introductions
Ana Jovanovic, Salford, United Kingdom

Patient perspectives: Experiences with FACEs
• Two people living with Fabry disease share their experiences of FACEs in a structured interview led by Ana Jovanovic
• Personal impact of FACEs on the people living with Fabry disease and their families
People living with Fabry disease and Ana Jovanovic, Salford, United Kingdom

Session 1: Overview of FACEs
• Presentation of an overview of FACEs, including:
  o The multisystemic nature of Fabry disease and preclinical manifestations
  o An introduction to FACEs, including use as a treatment outcome (not just as a clinical trial tool) and impact on clinical management
  o The effect of timely treatment in Fabry disease and the potential impact on FACEs (including morbidity and mortality)
  o Cardiac FACEs – impact on QoL and correlation with other measures, based on clinical experience
Peter Nordbeck, Würzburg, Germany

Session 2: Incidence of FACEs during migalastat treatment
• Presentation of FACE incidence in patients receiving migalastat, including:
  o Overview of previous data in the literature surrounding FACE incidence
  o Incidence of FACEs during a long-term post-hoc integrated analysis:
    ▪ Overall incidence and incidence by event category (renal, cardiac and cerebrovascular)
    ▪ Factors affecting time to first FACE
    ▪ Association between baseline variables and rate of FACEs (highlighting the importance of timely treatment initiation to manage disease)
  o followME baseline data – comparative history of FACEs and comorbidities across arms
Gere Sunder-Plassmann, Vienna, Austria

Live Q&A
Moderated Ana Jovanovic with Peter Nordbeck and Gere Sunder-Plassmann

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