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:
 - The multisystemic nature of Fabry disease and preclinical manifestations
 - An introduction to FACEs, including use as a treatment outcome (not just as a clinical trial tool) and impact on clinical management
 - The effect of timely treatment in Fabry disease and the potential impact on FACEs (including morbidity and mortality)
 - 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
 - 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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