Thursday, August 31st, 2023, 12.30-12.30, Hall Oranim

Addressing the needs of patients with Pompe disease – translating clinical trial data into real-world practice
Chair: Priya Kishnani (Durham, NC, United States)

1. To share the current evidence related to avalglucosidase alfa as an alternative therapy option for patients with Pompe disease
2. To discuss how patients, in real-world settings, are responding to avalglucosidase alfa

Introduction by Chair
Priya Kishnani (Durham, NC, United States)

Efficacy and safety of avalglucosidase alfa in participants with late-onset Pompe disease after 145 weeks' treatment during the COMET trial
Priya Kishnani (Durham, NC, United States)

A cohort summary: switching to avalglucosidase alfa in patients with infantile-onset and late-onset disease
Galit Tal (Haifa, Israel)

Real-world data: switching to avalglucosidase alfa and exploring the impact of home-infusion on the quality of life of patients with late-onset Pompe disease
Derralynn Hughes (London, United Kingdom)

Closing remarks & Q&A
Chair & All speakers

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