

Recordati, Tuesday August 30, 2022, 12.45-13.45, Hall Jerusalem

Optimization of long-term management of patients with organic acidaemia (propionic and methylmalonic acidurias)

Chairs: Sufin Yap, Sheffield, United Kingdom and Frank Rutsch, Münster, Germany

Introduction & Disease Overview

Sufin Yap, Sheffield, United Kingdom and Frank Rutsch, Münster, Germany

The metabolism and biochemistry we need to unravel
Urea and Krebs Cycle interplay & impairment in PA & MMA
François Feillet, Nancy – France

Real-world evidence with Carbaglu®

The PROspective Observational study of long-TErm Carbaglu® for the Treatment of PA & MMA study (PROTECT study)

Understanding the long-term Management of Organic Acidemia Patients with Carbaglu®: A Mixed Methods Approach

Sufin Yap, Sheffield, United Kingdom

Long-term management & treatment of patients with propionic and methylmalonic acidurias with Carbaglu® – Experience in UK, France & Germany

Sufin Yap, Sheffield, United Kingdom - Prof François Feillet, Nancy, France - Clemens Kamrath, Giessen, Germany

Three-year follow-up of patients with propionic and methylmalonic acidurias Long-term Carbaglu® vs liver transplant

Shirou Matsumoto, Kumamoto - Japan

Q&A & Closing Remarks

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