

PYRUVATE KINASE DEFICIENCY

Abstract: P1542

Comorbidities and complications across genotypes in adult patients with pyruvate kinase deficiency: Analysis from the Peak Registry

Andreas Glenthøj, Rachael F Grace, Eduard J van Beers, Joan-Lluís Vives Corrons, Bertil Glader, Kevin H M Kuo, Carl Lander, Dagmar Pospíšilová, Jean Williams, Yan Yan, Bryan McGee, Paola Bianchi

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Andreas Glenthøj

Abstract: P1546

ACTIVATE-KidsT: Mitapivat in children with pyruvate kinase deficiency who are regularly transfused

Rachael F Grace, Patrick D Tyler, Meredith Little, Penelope A Kosinski, Vanessa Beynon

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Rachael F Grace

Abstract: P1547

ACTIVATE-Kids: Mitapivat in children with pyruvate kinase deficiency who are not regularly transfused

Rachael F Grace, Patrick D Tyler, Meredith Little, Penelope A Kosinski, Vanessa Beynon

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Rachael F Grace

Abstract: P1548

Long-term treatment with oral mitapivat is associated with normalization of hemoglobin levels in patients with pyruvate kinase deficiency

Wilma Barcellini, Rachael F Grace, Hanny Al-Samkari, Andreas Glenthøj, Jennifer A Rothman, Marta Morado Arias, D Mark Layton, Oliver Andres, Melissa DiBacco, Peter Hawkins, Malia P Judge, Feng Tai, Jaime Morales-Arias, Vanessa Beynon, Eduard J van Beers

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Wilma Barcellini

Abstract: P1735

Improvements in patient-reported outcomes in mitapivat-treated patients with pyruvate kinase deficiency: A descriptive analysis from the phase 3 ACTIVATE trial

Kevin H M Kuo, Rachael F Grace, Eduard J van Beers, Wilma Barcellini, Andreas Glenthøj, Susanne Holzhauer, Parija Patel, Vanessa Beynon, Junlong Li, Erin Zagadailov, Hanny Al-Samkari

Acceptance Status: Poster presentation

Session Title: Poster session; Quality of life, palliative care, ethics and health economics

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Kevin H M Kuo

Abstract: P1543

Mitapivat decreases the need for transfusions secondary to poorly tolerated anemia and acute events compared to placebo in patients with pyruvate kinase deficiency who are not regularly transfused

Hanny Al-Samkari, Jaime Morales-Arias, Rengyi Xu, Vanessa Beynon, Rachael F Grace

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Hanny Al-Samkari

Abstract: P1544

Bone mineral density remains stable in pyruvate kinase deficiency patients receiving long-term treatment with mitapivat

Hanny Al-Samkari, Rachael F Grace, Andreas Glenthøj, Oliver Andres, Wilma Barcellini, Frédéric Galactéros, Kevin H M Kuo, D Mark Layton, Marta Morado Arias, Vip Viprakasit, Yan Dong, Feng Tai, Liz Grekas, Karishma Khoja, Sarah Gheuens, Bryan McGee, John B Porter, Eduard J van Beers

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Hanny Al-Samkari

Abstract: P1545

Durability of hemoglobin response and reduction in transfusion burden is maintained over time in patients with pyruvate kinase deficiency treated with mitapivat in a long-term extension study

Rachael F Grace, Andreas Glenthøj, Wilma Barcellini, Madeleine Verhovsek, Jennifer A Rothman, Marta Morado Arias, D Mark Layton, Oliver Andres, Frédéric Galactéros, Eduard J van Beers, Koichi Onodera, Vip Viprakasit, Satheesh Chonat, John B Porter, Malia P Judge, Penelope A Kosinski, Peter Hawkins, Sarah Gheuens, Rengyi Xu, Bryan McGee, Vanessa Beynon, Hanny Al-Samkari

Acceptance Status: Poster presentation

Session Title: Poster session; Enzymopathies, membranopathies and other anemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Rachael F Grace

Abstract: P1562

Characterizing iron overload by age in patients diagnosed with pyruvate kinase deficiency – A descriptive analysis from the Peak Registry

Paola Bianchi, Rachael F Grace, Joan-Lluís Vives Corrons, Bertil Glader, Andreas Glenthøj, Hitoshi Kanno, Kevin H M Kuo, Carl Lander, D Mark Layton, Dagmar Pospíšilová, Vip Viprakasit, Jean Williams, Yan Yan, Bryan McGee, Eduard J van Beers

Acceptance Status: Poster presentation

Session Title: Poster session; Iron metabolism, deficiency and overload

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Paola Bianchi

Abstract: P1565

Mitapivat improves ineffective erythropoiesis and reduces iron overload in patients with pyruvate kinase deficiency

Eduard J van Beers, Hanny Al-Samkari, Rachael F Grace, Wilma Barcellini, Andreas Glenthøj, Malia P Judge, Penelope A Kosinski, Rengyi Xu, Vanessa Beynon, Bryan McGee, John B Porter, Kevin H M Kuo

Acceptance Status: Poster presentation

Session Title: Poster session; Iron metabolism, deficiency and overload

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Eduard J van Beers

Abstract: PB2247

Pharmacokinetic modeling and simulation to support mitapivat dose selection used in pediatric phase III studies

Ophelia Yin, Kyle Baron, John Mondick, Meredith Little, Patrick D Tyler, Vanessa Beynon

Acceptance Status: Publication only

Abstract: PB2359

Healthcare resource utilization of adult patients with pyruvate kinase deficiency: A real world study using US veterans health administration data

Erin Zagadailov, Viviana Garcia-Horton, Sara Higa, Lizheng Shi

Acceptance Status: Publication only

Abstract: PB2345

Adapting patient educational approaches in pyruvate kinase deficiency: Europe and North America findings from the AAC patient and caregiver survey

Wilma Barcellini, Rachael F Grace

Acceptance Status: Publication only

The AAC was created and activities funded by Agios Pharmaceuticals, Inc.

SICKLE CELL DISEASE

Abstract: P1501

Follow-up results of a phase 2 study assessing the safety and efficacy of mitapivat treatment, an oral pyruvate kinase activator, for up to 60 weeks in subjects with sickle cell disease

Myrthe J van Dijk, Minke A E Rab, Brigitte A van Oirschot, Jennifer Bos, Cleo Derichs, Myrthe J van Dijk, Minke A E Rab, Brigitte A van Oirschot, Jennifer Bos, Cleo Derichs, Anita W Rijnveld, Marjon H Cnossen, Erfan Nur, Bart J Biemond, Marije Bartels, Judith J M Jans, Wouter W van Solinge, Roger E G Schutgens, Richard van Wijk, Eduard J van Beers

Acceptance Status: Poster presentation

Session Title: Poster session; Sickle cell disease

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Myrthe J van Dijk

Presentation is supported but not sponsored by Agios

Abstract: P1492

Pharmacodynamic effects of AG-946, a highly potent novel activator of pyruvate kinase, in ex vivo treatment of red blood cells from sickle cell disease patients

Minke A E Rab, Myrthe J van Dijk, Jennifer Bos, Brigitte A van Oirschot, Johan Gerrits, Penelope A Kosinski, Charles Kung, Judith Jans, Eduard J van Beers, Lenny Dang, Richard van Wijk

Acceptance Status: Poster presentation

Session Title: Poster session; Sickle cell disease

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Minke A E Rab

Presentation is supported but not sponsored by Agios

THALASSEMIA

Abstract: P1522

Long-term efficacy and safety of the oral pyruvate kinase activator mitapivat in adults with non-transfusion-dependent alpha- or beta-thalassemia

Kevin H M Kuo, D Mark Layton, Ashutosh Lal, Hanny Al-Samkari, Joy Bhatia, Penelope A Kosinski, Bo Tong, Megan Lynch, Katrin Uhlig, Elliott P Vichinsky

Acceptance Status: Poster presentation

Session Title: Poster session; Thalassemias

Date/Time: Friday, June 10, 2022; 16:30 – 17:45 CEST

Presenting Author: Kevin H M Kuo

Abstract: PB2233

Systematic literature review of health-related quality of life burden in patients across the spectrum of thalassemia

Khaled M Musallam, Lydia Vinals, Keely Gilroy, Maria Rizzo, Louise Lombard

Acceptance Status: Publication only

OTHER

Abstract: S273

Evidence of noninferiority of mitapivat versus splenectomy in murine hereditary spherocytosis

Alessandro Mattè, Antonio Recchiuti, Enrica Federti, Penelope A Kosinski, Veronica Riccardi, Iana Iatcenko, Lenny Dang, Achille Iolascon, Roberta Russo, Carlo Brugnara, Anne Janin, Christophe Leboeuf, Narla Mohandas, Lucia De Franceschi

Acceptance Status: Oral presentation

Session Title: Oral sessions; Thalassemia and rare anemias

Date/Time: Saturday, June 11, 2022; 16:30 – 17:45 CEST

Location: Hall A2 – A3

Presenting Author: Alessandro Mattè

Presentation is supported but not sponsored by Agios

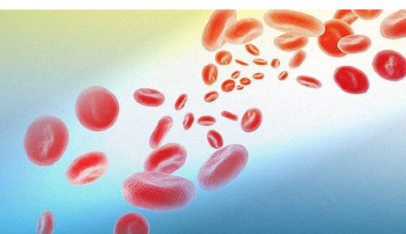
Satellite Symposium

DURING THE 2022 EUROPEAN HEMATOLOGY ASSOCIATION CONGRESS



Pyruvate kinase activation: Latest clinical trial results across hemolytic anemias

Thursday, June 9
19:15–20:45 CEST



Please join us for this informational session on pyruvate kinase activation in hemolytic anemias

This hybrid symposium will be held in room **Strauss 3**.

Our invited speakers will review the latest clinical trial data on mitapivat, the first disease-modifying therapy for hemolytic anemia in pyruvate kinase deficiency, and will provide an overview of its potential therapeutic roles in thalassemia and sickle cell disease.

Speakers

Maria Domenica Cappellini, MD, FRCP, FACP, Chair

University of Milan, Italy

Hanny Al-Samkari, MD

Massachusetts General Hospital, Harvard Medical School, Boston, USA

Kevin H M Kuo, MD, MSc, FRCPC

University of Toronto, Toronto General Hospital, Ontario, Canada

Eduard J van Beers, MD, PhD

Benign Hematology Center, Van Creveldkliniek, University Medical Center Utrecht, University Utrecht, The Netherlands