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, Joann-Luis Vives Corrons, Bertil Glader, Kevin H M Kuo, Carl Lander, Dagmar Pospišilová, 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-Kids: Mitapivat in children with pyruvate kinase deficiency who are regularly transfused
Rachael F Grace, Patrick O 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 O 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 Rathman, Marla Morada Arias, D Mark Layton, Olivier 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, Renyi 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, Marla Morada Arias, Vip Viprakasit, Yan Dong, Feng Tai, Liz Grekas, Karisma 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 Verhavsek, Jennifer A Rathman, Marla Morada Arias, D Mark Layton, Olivier 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, Renyi 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
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, Renyi 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

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**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


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*

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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*

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**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

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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

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**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 Latcenko, Lenny Dang, Achille Iolascon, Roberta Russo, Carlo Brugnara, Anne Janin, Christophe Leboeuf, Noria 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*