

## PK DEFICIENCY

**Abstract:** S270

ACTIVATE: A Phase 3, Randomized, Multicenter, Double-Blind, Placebo-Controlled Study of Mitapivat in Adults With Pyruvate Kinase Deficiency Who Are Not Regularly Transfused

Al-Samkari H, Galactéros F, Glenthøj A, Rothman JA, Andres O, Grace RF, Arias MM, Layton DM, Onodera K, Verhovsek M, Barcellini W, Judge MP, Beynon V, Xu E, Hawkins P, Zagadailov E, Gheuens S, van Beers EJ

**Acceptance Status:** Oral Presentation

**Session Title:** Changing the scene on congenital anemias

**Abstract:** S271

Activate-T: A Phase 3, Open-Label, Multicenter Study of Mitapivat in Adults With Pyruvate Kinase Deficiency Who Are Regularly Transfused

Glenthøj A, van Beers EJ, Al-Samkari H, Viprakasit V, Kuo KHM, Galactéros F, Chonat S, Porter J, Gheuens S, Beynon V, Xu E, Hawkins P, Zagadailov E, Oluyadi A, Barcellini W

**Acceptance Status:** Oral Presentation

**Session Title:** Changing the scene on congenital anemias

**Abstract:** EP696

Bone Mineral Density Is Stable in Adults With Pyruvate Kinase Deficiency Receiving Long-Term Treatment With Mitapivat

Al-Samkari H, van Beers EJ, Barcellini W, Galactéros F, Glader B, Kuo KHM, Layton DM, Ravindranath Y, Yaish H, Dong Y, Tai F, Hawkins P, Beynon V, Gilroy K, Grace RF

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

**Abstract:** EP692

Early-Onset Osteopenia and Osteoporosis in Patients With Pyruvate Kinase Deficiency

Al-Samkari H, Grace RF, Glenthøj A, Andres O, Barcellini W, Galactéros F, Kuo KHM, Layton DM, Arias MM, Viprakasit V, Dong Y, Tai F, Hawkins P, Gheuens S, Bowden C, Porter J, van Beers EJ

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

**Abstract:** EP691

Baseline Characteristics by Age of a Global Cohort of Patients Diagnosed With Pyruvate Kinase Deficiency – A Descriptive Analysis From the PEAK Registry

Bianchi P, van Beers EJ, Vives Corrons J-L, Glader B, Glenthøj A, Kanno H, Kuo KHM, Lander C, Layton DM, Pospíšilová D, Viprakasit V, Williams J, Yan Y, McGee B, Grace RF

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

**Abstract:** EP710

Mortality Among Veterans With a Diagnosis of Pyruvate Kinase Deficiency: A Real-World Study Using US Veterans Health Administration Data (Encore)

Zagadailov E, Boscoe A, Garcia-Horton V, Shi S, Liu S, Shi L, Macaulay D

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

**Abstract:** EP1194

The Lifetime Economic Burden of Pyruvate Kinase Deficiency in the United States

Su W, van Stiphout J, Kuo KHM, Grace RF, Chonat S, Yaish H, Ravindranath Y, Zagadailov E

**Acceptance Status:** e-Poster

**Topic:** Quality of life, palliative care, ethics and health economics

**Abstract:** EP686

Rare Anemia Disorders European Epidemiological Platform (RADeep): Distribution of Patients Affected by Pyruvate Kinase Deficiency in Europe

del Mar Mañú-Pereira M, Bianchi P, Van Wijk R, Glenthøj A

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

*Presentation is supported but not sponsored by Agios.*

**Abstract:** EP712

Usefulness of NGS Techniques for Pyruvate Kinase Deficiency (PKD) Diagnosis in Spain

Morado M, Nieto JM, Del Orbe Barreto R, González Fernández FA, Arrizabalaga B, Villegas A

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

*Presentation is supported but not sponsored by Agios.*

**Abstract:** EP711

Feasibility Study: Using Dried Blood Spots as a DNA Source to Screen for Pyruvate Kinase Causing Variants

Charlton J, McGuire C, Perera R, Laas C, Rakhit C, Rees D, Clark B

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

*Presentation is supported but not sponsored by Agios.*

The safety and efficacy of the agents and uses under investigation have not been established. There is no guarantee that the agents will receive health authority approval or become commercially available in any country for the uses being investigated.

## THALASSEMIA

**Abstract:** S267

Results From a Phase 2, Open-Label, Multicenter Study of the Oral Pyruvate Kinase Activator Mitapivat in Adults With Non-Transfusion-Dependent Alpha- or Beta-Thalassemia

Kuo KHM, Layton DM, Lal A, Al-Samkari H, Bhatia J, Tong B, Lynch M, Uhlig K, Vichinsky EP

**Acceptance Status:** Oral Presentation

**Session Title:** Changing the scene on thalassemias

**Abstract:** PB1805

ENERGIZE and ENERGIZE-T: Two Phase 3, Randomized, Double-Blind, Placebo-Controlled Studies of Mitapivat in Adults With Non-Transfusion-Dependent or Transfusion-Dependent Alpha- or Beta-Thalassemia

Kuo KHM, Layton DM, Al-Samkari H, Kattamis A, Sheth S, Taher A, Viprakasit V, Chamberlain CX, Czaplá L, Gheuens S, Jiang L, Lynch M, Tong B, Uhlig K, Cappellini MD

**Acceptance Status:** Publication Only

**Topic:** Thalassemias

**Abstract:** EP1318

Global Thalassemia Epidemiology: A Systematic Literature Review

Chamberlain CX, Zagadailov E, Kistler K, Colby JA, Ruiz K

**Acceptance Status:** e-Poster

**Topic:** Thalassemias

## HEREDITARY SPHEROCYTOSIS

**Abstract:** EP695

Mitapivat Ameliorates Red Cell Features and Improves Anemia in Band 4.2-/- Mice, a Model of Hereditary Spherocytosis

Matte A, Recchiuti A, Federti E, Kung C, Wilson AB, Pomilio A, Kosinski PA, Riccardi V, Iatchenko I, Dang L, Iolascon A, Russo R, Brugnara C, Janin A, Leboeuf C, Mohandas N, De Franceschi L

**Acceptance Status:** e-Poster

**Topic:** Enzymopathies, membranopathies and other anemias

*Presentation is supported but not sponsored by Agios.*

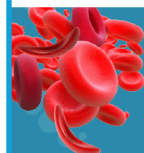
All presentations will be available on the on-demand Virtual Congress platform as of:

**Friday, June 11**

**09:00 CEST**

*Presentations will be accessible until August 15, 2021*

UPDATE IN HEMATOLOGY (UIH) SESSION  
DURING THE 2021 EUROPEAN HEMATOLOGY ASSOCIATION CONGRESS



### Pyruvate Kinase Activation: Update on Clinical Trial Results Across Hemolytic Anemias

Thursday, June 17 18:00 - 19:30 CEST

Invitation

**Join this informational session on pyruvate kinase activation in a range of hemolytic anemias**

This session will review pyruvate kinase activation through mitapivat and how it is currently being evaluated as a potential therapeutic option across a number of clinical trials in patients with pyruvate kinase deficiency, thalassemia, and sickle cell disease.

### Speakers

**Eduard van Beers, MD, PhD, Chair**

Van Creveldkliniek  
University Medical Center Utrecht  
Utrecht, The Netherlands

**Hanny Al-Samkari, MD**

Division of Hematology  
Massachusetts General Hospital  
Harvard Medical School  
Boston, United States

**Kevin HM Kuo, MD, MSc, FRCPC**

University of Toronto  
Toronto General Hospital  
Ontario, Canada