

IVOSIDENIB (AG-120)

Abstract: 625

Ivosidenib Improves Overall Survival Relative to Standard Therapies in Relapsed or Refractory Mutant *IDH1* AML: Results from Matched Comparisons to Historical Controls

Paschka P, Dombret H, Thomas X, Recher C, Chantepie S, Montesinos Fernandez P, Acuña-Cruz E, Vyas P, Kreuzer K-A, Heuser M, Metzeler KH, Dennis M, Quesnel B, Hunault-Berger M, Mohty M, Pigneux A, de Botton S, Weber D, Döhner K, Milkovich G, Reitan J, MacDonald SC, Casso D, Storm M, Liu H, Kapsalis SM, Attar EC, Winkler T, Döhner H

Acceptance Status: Oral Presentation

Session Title: 903. Health Services Research—Malignant Conditions (Myeloid Disease): Treatment and Publication Patterns in Myeloid Malignancies

Abstract: 2900

Longitudinal Molecular Profiling in Patients with *IDH1*-Mutant Newly Diagnosed Acute Myeloid Leukemia Treated with Ivosidenib

Choe S, Wang H, Roboz GJ, DiNardo CD, Stein EM, Mims AS, Prince GT, Watts JM, Fan B, Nejad P, Zhang V, Liu H, Attar EC, Wu B, Stone RM

Acceptance Status: Poster

Session Title: 617. Acute Myeloid Leukemia: Biology, Cytogenetics, and Molecular Markers in Diagnosis and Prognosis: Poster III

Abstract: 2814

AGILE: Phase 3, Double-Blind, Randomized, Placebo-Controlled Study of Ivosidenib in Combination with Azacitidine in Adults with Newly Diagnosed Acute Myeloid Leukemia and an *IDH1* Mutation

Montesinos Fernandez P, Recher C, Zarzycka E, Doronin V, McCulloch D, Vives S, Calado RT, Jang JH, Miyazaki Y, Wang J, Gianolio DA, Daigle SR, Winkler T, Zhang V, Paschka P

Acceptance Status: Poster

Session Title: 613. Acute Myeloid Leukemia: Clinical Studies: Poster III

Abstract: 1943

Molecular Characterization of Clinical Response and Relapse in Patients with *IDH1*-Mutant Newly Diagnosed Acute Myeloid Leukemia Treated with Ivosidenib and Azacitidine

Daigle SR, Choe S, DiNardo CD, Stein AS, Stein EM, Fathi AT, Frankfort O, Schuh AC, Döhner H, Martinelli G, Patel PA, Raffoux E, Tan P, Zeidan AM, de Botton S, Stone RM, Frattini M, Franovic A, Xu E, Winkler T, Wu B, Vyas P

Acceptance Status: Poster

Session Title: 615. Acute Myeloid Leukemia: Commercially Available Therapy, excluding Transplantation: Poster II

ENASIDENIB (AG-221)

Abstract: 2402

A Phase I Study of the *IDH2* Inhibitor Enasidenib as Maintenance Therapy for *IDH2*-Mutant Myeloid Neoplasms Following Hematopoietic Cell Transplantation

Fathi AT, Li S, Soiffer RJ, Levis MJ, Mims AS, Devine SM, Defilipp Z, El-Jawahri A, McAfee SL, Spitzer TR, Frigault MJ, Dey BR, Amrein PC, Hobbs G, Brunner AM, Hock HR, Narayan R, Knight LW, Kelley D, Bottoms AS, Brown JL, Del Rio CJ, Vanderklis JE, Danielson C, Saylor ML, Hunnewell CL, Pery LH, Wahl JL, Breton E, Graubert TA, Chen YB

Acceptance Status: Poster

Session Title: 723. Clinical Allogeneic and Autologous Transplantation: Late Complications and Approaches to Disease Recurrence: Poster II

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.

PYRUVATE KINASE DEFICIENCY

Abstract: 1679

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

Al-Samkari H, Grace RF, Glenthoej A, Andres O, Barcellini W, Galactéros F, Kuo KHM, Layton DM, Morado M, Viprakasit V, Dong Y, Tai F, Hawkins P, Gheuens S, Bowden C, Porter JB, van Beers E

Acceptance Status: Poster

Session Title: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster II

Abstract: 2583

Baseline Characteristics of Patients in Peak: A Global, Longitudinal Registry of Patients with Pyruvate Kinase Deficiency

Grace RF, Boscoe AN, Bowden C, Glader B, Kanno H, Layton DM, van Beers E, Vives Corrons J-L, Yan Y, Bianchi P

Acceptance Status: Poster

Session Title: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III

Abstract: 1627

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

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

Acceptance Status: Poster

Session Title: 904. Outcomes Research—Non-Malignant Conditions: Poster I

MITAPIVAT (AG-348)

Abstract: 2600

Proof of Concept for the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Thalassemia: Interim Results from an Ongoing, Phase 2, Open-Label, Multicenter Study

Kuo KHM, Layton DM, Lal A, Al-Samkari H, Tai F, Lynch M, Uhlig K, Vichinsky EP

Acceptance Status: Poster

Session Title: 112. Thalassemia and Globin Gene Regulation: Poster III

Abstract: 681

Phase 1 Multiple Ascending Dose Study of Safety, Tolerability, and Pharmacokinetics/Pharmacodynamics of Mitapivat (AG-348) in Subjects with Sickle Cell Disease

Xu J, Conroy A, Frey I, Nichols N, Menapace L, Tumburu L, Lequang T, Li Q, Dunkelberger E, Henry E, Cellmer T, Iyer V, Mangus H, Kung C, Dang L, Kosinski P, Hawkins P, Jeffries N, Eaton W, Thein S

Acceptance Status: Oral Presentation

Session Title: 114. Hemoglobinopathies, Excluding Thalassemia—Clinical: Novel Treatments for Sickle Cell Disease

Presentation is supported but not sponsored by Agios.

Abstract: 84

The Pyruvate Kinase Activator Mitapivat Ameliorates Anemia and Prevents Iron Overload in a Mouse Model of Hereditary Spherocytosis

Matte A, Wilson A, Federti E, Kung C, Kosinski PA, Riccardi V, Iatchenko I, Dang L, Lebouef C, Janin A, Brugnara C, Naria M, De Franceschi L

Acceptance Status: Oral Presentation

Session Title: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron

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