

## **616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Novel Targeted and Immune-based Approaches in the Treatment of AML**

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[Remissions of Acute Myeloid Leukemia and Blastic Plasmacytoid Dendritic Cell Neoplasm Following Treatment with CD123-Specific CAR T Cells: A First-in-Human Clinical Trial](#)

**Lihua Budde, MD, PhD<sup>1</sup>**, Joo Y Song, MD<sup>1\*</sup>, Young Kim, MD<sup>1\*</sup>, Suzette Blanchard, PhD<sup>1\*</sup>, et al.

**The authors of the study conclude that:**

*In this first-in-human clinical trial of CD123CAR T cell therapy, we have demonstrated the feasibility and safety of targeting CD123. We have also observed promising anti-leukemic activity in both AML and BPDCN. Importantly, no myeloablative effects have been observed, supporting further testing of this immunotherapeutic strategy in both transplant eligible and ineligible patients.*

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[Targeting the Transcriptional Addiction of Leukemia Stem Cells By a New Class of Protein Kinase Inhibitors](#)

**Yinon Ben-Neriah, MD, PhD<sup>1\*</sup>**, Avanthika Venkatachalam, MSc<sup>1\*</sup>, Avner Fink, PhD<sup>1\*</sup>, Eric Hung,

MSc<sup>1\*</sup>, J et al.

**The authors of the study conclude that:**

*We developed a new class of small molecule inhibitors that co-targets CKI $\alpha$  and P-TEFb. These inhibitors have unique pharmacologic properties: short-term kinase inhibition results in long-term disruption of SE activity. Shutdown of leukemic super-enhancers in synergy with robust p53 activation compromises leukemic cells and stem cells addicted to SE-driven transcription. These features explain the powerful and specific anti-leukemic therapeutic effects of this new class of inhibitors in-vivo.*

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[Preliminary Results from a Phase Ib Study Evaluating BCL-2 Inhibitor Venetoclax in Combination with MEK Inhibitor Cobimetinib or MDM2 Inhibitor Idasanutlin in Patients with Relapsed or Refractory \(R/R\) AML](#)

Naval Daver, MD<sup>1</sup>, Daniel A. Pollyea, MD<sup>2,3</sup>, Karen W.L. Yee, MD<sup>4\*</sup>, Pierre Fenaux, MD, PhD<sup>5</sup>, et al.

**The authors of the study conclude that:**

*Preliminary results show that VEN plus cobimetinib or idasanutlin can be administered with appropriate risk mitigation measures for GI toxicity and early evidence of clinical activity in R/R AML pts. Dose finding is ongoing and the MTD for both combinations has not yet been determined. Preliminary ORR for the VEN 600 mg + idasanutlin 200 mg cohort was encouraging at 38%. Safety, PK and efficacy data will be updated at the time of presentation.*

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[TAK-243 Is a Selective UBA1 Inhibitor That Displays Preclinical Activity in Acute Myeloid Leukemia \(AML\)](#)

**Samir H. Barghout, BSPHarm, MSc<sup>1,2</sup>**, Parasvi Patel, BSc (Hons)<sup>1,2\*</sup>, Xiaoming Wang<sup>1\*</sup>, G. Wei Xu<sup>1\*</sup>, S et al.

**The authors of the study conclude that:**

*TAK-243 is a potent and selective UBA1 inhibitor that displays preferential activity towards AML cells over normal hematopoietic cells. Acquired mutations affect drug binding and may be a clinically relevant mechanism of resistance. These data support conducting a clinical trial of TAK-243 in patients with AML.*

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[Phase 2 Study of Combination of Cytarabine, Idarubicin, and Nivolumab for Initial Therapy of Patients with Newly Diagnosed Acute Myeloid Leukemia](#)

**Farhad Ravandi, MBBS<sup>1</sup>**, Naval Daver, MD<sup>2</sup>, Guillermo Garcia-Manero, MD<sup>2</sup>, Christopher B Benton, MD<sup>3</sup>, et al.

**The authors of the study conclude that:**

*Addition of nivolumab to ara-C and anthracycline induction chemotherapy is feasible and safe in younger pts with AML. Among the pts proceeding to alloSCT the risk of GVHD is not significantly increased.*

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[Selinexor in Combination with Cladribine, Cytarabine and G-CSF for Relapsed or Refractory AML](#)

**Geoffrey L. Uy, MD<sup>1</sup>**, Michael P Rettig, PhD<sup>1</sup>, Theresa Fletcher<sup>1\*</sup>, Peter A Riedell, MD<sup>2</sup>, et al.

**The authors of the study conclude that:**

*That selinexor + CLAG is highly active in patients with relapsed or refractory AML and has encouraging rates of CR. Furthermore, the combination serves as a bridge which allows a high percentage of patients to undergo allogeneic hematopoietic cell transplantation.*