CPI-613 has Activity in AML, Patient #4

Patient #4, a 65 y/o male diagnosed with AML with normal karyotype in 2004. After standard therapy achieved CR1. Relapsed in 2008, received new AML induction (7+3). Transplanted in 2010 while in CR2. Delayed count recovery, red cell and platelet transfusion dependent. Three months post transplant found to have 8.5% cells with 7q-. Marrow 6 months post transplant showing 41% morphologically leukemic cells.

Conclusions
CPI-613 is a first in class non-redox active lipoate derivative currently under study in the laboratory and in a phase I clinical trial for patients with relapsed or refractory hematologic malignancies. To date we have shown:

- CPI-613 is active in vitro against several human leukemia cell lines with IC50 values in the low nM range.
- CPI-613 displays strong synergy with tyrosine kinase inhibitors in ALL and AML cell lines.
- CPI-613 displays synergy with the anthracycline doxorubicin in vitro and in vivo.
- CPI-613 inhibits fatty acid synthesis

No DLT identified even at a dose of 2940 mg/m².

CPI-613 has activity in multiple hematologic malignancies. Of the eight eligible patients with a diagnosis of AML or MDS, one achieved a CR, one a morphologic leukemia free state and two had stable disease for an overall response rate of 38%.

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