Highly promising AML therapy, curative in mouse model

Reference No: B78209

CHALLENGE

Acute myeloid leukemia (AML) is one of the cancers with the worst prognosis. Current AML treatment is mostly based on highly aggressive chemotherapy or stem cell transplant with substantial side effects and high financial burden. Lasting remissions after disease relapse following initial chemotherapy remain highly unlikely, rendering most patients incurable in this setting. Novel treatment options (TKI, apoptosis inducers, epigenetics, cellular Tx) so far fail to induce lasting cures.

Resistant leukemic stem cells represent the reservoir for relapse, rendering novel treatment options for this critical cellular population highly attractive to improve the prognosis for patients diagnosed with AML.

INNOVATION

The innovative agent rapidly and specifically activates programmed cell death in both leukemic blasts and leukemic stem cells, strongly reducing the possibility of a leukemia relapse. Specifically, the innovation lies in

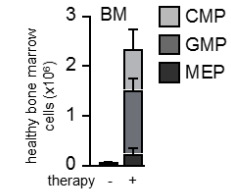
(i) the selective activation of programmed cell death in leukemic cells only. Aberrations in the composition of receptor-proximal kinases present only in leukemic cells, render healthy bone marrow cells unaffected by the innovative agent. => **High selectivity**

(ii) the selective propagation of healthy hematopoietic cells in the bone marrow. The new agent boosts normal hematopoietic stem cells by inducing a mild inflammatory milieu driving progenitor cell expansion. This process results in the expulsion of leukemic stem cells from the bone marrow niche and a near complete elimination of stem cell capacity. => **High efficacy**

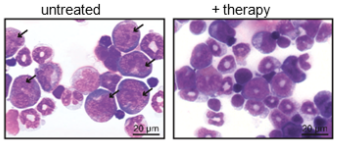
(iii) its little to no side effects in immune-competent murine models of AML. The innovative agent even improves normal blood cell counts upon treatment (see (ii)). => **Broad therapeutic window**

Advantages of the innovative agent:

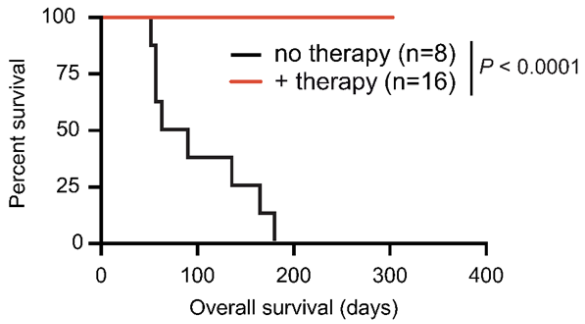
- Fast and specific elimination of leukemia blasts and stem cells
- Highly improved survival and fitness in mouse model
- Large percentage of animals are actually cured in mouse model
- Applicable for most AML patients
- Low side effects compared to conventional therapies



Boosting healthy hematopoiesis



Elimination of leukemia blasts and stem cells



Strongly improved survival (mouse model)

COMMERCIAL OPPORTUNITIES

Stand-alone or add-on therapy for AML.

DEVELOPMENT STATUS

Mouse efficacy and toxicity data, ex vivo human (patient blood cell) data, compound should likely be optimized regarding half-life and stability.

REFERENCES:

Publication expected in early 2022.

BayPAT

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IP rights:
EP, US, CA, JP pending (PCT application was in 2020)

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