HKU Identifies Homoharringtonine as Effective Treatment for FLT3 Mutant Acute Myeloid Leukaemia for the First Time in the World

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Speakers

Professor Anskar Leung Yu-hung
Li Shu Fan Medical Foundation Professor in Haematology
Clinical Professor of Department of Medicine
Li Ka Shing Faculty of Medicine, HKU

Professor Kwong Yok-lam
Chui Fook-Chuen Professorship in Molecular Medicine
Chair Professor of Haematology and Haematological Oncology
Department of Medicine
Li Ka Shing Faculty of Medicine, HKU

Mr Stephen Lam Sze-yuen
MBBS / PhD student
Li Ka Shing Faculty of Medicine, HKU
Acute Leukaemia

- Symptoms include anaemia, fever, infection and easy bruising or bleeding.
- Patients have to undergo blood tests bone marrow biopsy for immunological cancer, chromosome and gene analysis.
- Divided into two categories: acute myeloid and lymphoid leukaemia.
Acute myeloid leukemia (AML) is characterised by an abnormal increase in myeloblasts and patients die of bleeding and infection.
Conventional treatment comprises chemotherapy and allogeneic haematopoietic stem cell transplantation. Relapsed and refractory disease is a major cause of failure.
Molecular Profiling and Drug Screening led to Personalized Therapeutic Strategy and Biomarker Development.

Heterogeneity between and within individual AML

Molecular Profiling

Patients

Genetic mutations

Dose Response and unbiased clustering

Personalized therapeutic strategy and biomarker development

Validation by clinical trials
FLT3-ITD AML

- 30% AML patients are diagnosed FLT3 mutant AML, resulting in cancer cell hyperactivity and the emergence of drug resistance.

- Conventional treatments including chemotherapy, allogeneic haematopoietic stem cell transplantation and FLT3 inhibitor are not satisfactory for AML patients carrying FLT3 mutation. Less than 10% of the patients can survive long-term.
Homoharringtoninine (HHT)

- HHT is a drug derived from a natural plant *Cephalotaxus fortunei*, which can be found in Shaanxi, Henan, Hubei, Zhejiang, Sichuan of Mainland China.

- HHT has been used for cancer treatment in China since the 1970s.

- It is used indiscriminately in AML and there is no biomarker to identify the sensitive subtypes.

- As a result, the overall response has been unsatisfactory.
HHT Treatment Results

Combination of homoharringtonine and FLT3 inhibitor resulted in clearance of myeloblasts in 20 out of 24 patients

Before treatment

After treatment (3 weeks)
Implications

- The research team discovered that HHT and FLT3 inhibitor in combination could eradicate leukaemia cells in patients with FLT3-ITD AML and prolonged their leukaemia free and overall survivals.

- The treatment was associated with minimal side effects.

- The research was recognised internationally and has set a new paradigm for the development of novel therapeutic agents in relapsed or refractory AML based on *in vitro* drug screening.
Future Study Plans

• The platform arising in this study will be improved and modified for use in other AML subtypes with poor response to conventional treatment.

• Novel therapeutic agents currently in clinical trials for cancer therapy with a potential in AML will be included in the drug library for screening.

• The revised platform and drug library will likely generate novel information about biomarkers predictive of clinical drug response.
Patient’s Sharing
Q & A Session
Thank you