



SUPPORTING INNOVATION AND TECHNOLOGY TRANSFER IN ONCOLOGY

THERAPY

UnGRASP

Therapeutic inhibition of GRASP55/JAM-C pathway in Acute Myeloid Leukemia through protein/protein interaction inhibition



CONTEXT & BACKGROUND

Acute Myeloid Leukemia (AML) is a disease with poor prognosis for which few new therapeutic agents have been approved over the last decades. The disease is driven by stepwise accumulation of genetic alterations in hematopoietic stem and progenitor cells (HSPC) which results in intratumoral heterogeneity due to the co-existence of pre-leukemic cells, leukemic blasts and leukemic stem cells (LSC). Clonal expansion of selected subclones depends on the nature of the mutation(s) and is controlled, at least in part, by extrinsic signals that are still poorly understood. We have identified a pathway involved in the maintenance of LSC that repopulate the leukemia after chemotherapy. Beyond the value of the identified pathway as poor prognosis marker in AML (patent EP15305471.3), we have identified a small compound inhibiting protein/protein interaction and interfering with such a pathway (patent EP15306260.9).



INNOVATIVE COMPONENT & TECHNOLOGY

Protein/protein interaction inhibitor
New pathway



OBJECTIVES

Develop first in class compounds belonging to protein/protein interaction inhibitors and targeting LSC through the newly identified pathway



DEVELOPMENT & MATURATION STAGE

One compound identified and tested in preclinical model



TARGET POPULATION

Poor prognosis AML patients stratified on the basis of our biomarker (represent around 50% of the patients). May be indicated in other haematological malignancies and in Alzheimer's disease

SCOPE

Therapeutic target for
Acute Myeloid Leukemia

KEYWORDS

Targeted Therapy,
Biomarker, Drug
resistance, Protein/
protein interaction
inhibitors





TARGET PROFILE

Orally available adjuvant treatment for AML patients.



STRENGTHS & COMPETITIVE ADVANTAGES

Originality of the target
Strong expertise in the field
Excellent translational research collaborative network
Large recruitment of patients in hematology at Institut Paoli Calmettes



INDUSTRIAL APPLICATIONS & OPPORTUNITIES

Strong partnership with a translational research group in leukemic disease. Opportunity to work with a collaborative group involved at all stages from target identification and validation, compound discovery and validation in preclinical models, to early 2 clinical trial if applicable.



INTELLECTUAL PROPERTY & PATIENT CO-OWNER(S)

EP15305471.3 Inserm / Aix-Marseille Université
EP15306260.9 Inserm / Aix-Marseille Université
EP2012/051793 Inserm / Aix-Marseille Université

