MYELODYSPLASTIC SYNDROMES-ACUTE MYELOID LEUKEMIA MOON SHOT

INTRODUCTION

Myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML) are a complex group of disorders affecting the bone marrow. About 10,000 people receive an MDS diagnosis annually and an estimated 13,000 new cases of AML are detected. Half of all MDS and AML patients die of complications of their disease. In MDS, the bone marrow produces too few blood cells. AML involves an overgrowth of immature blood cells so there aren't enough mature blood cells to prevent anemia, infection or bleeding.

- The origins of AML and MDS and the causes of disease progression are poorly understood. Both are driven by varied genomic abnormalities, which hinder treatment. There are at least 20 types of MDS.

- MDS and AML are separate malignancies that develop along a continuous spectrum ranging from early stage MDS, which might never progress or might develop into advanced MDS or AML.

- AML can originate from advanced MDS, from damage to bone marrow from previous treatment for other cancers or emerge from unknown origins. MD Anderson researchers have found these to be three different versions of AML.

- Treatment options are limited mainly to untargeted chemotherapy combinations and blood stem cell transplants.

KEY PROJECTS

MD Anderson's MDS/AML moon shot will address all of the above aspects of these cancers. It starts with a critical mass of expertise and resources – the world's leading and largest MDS, AML and blood stem cell transplant programs; global leadership in clinical trial development, a tissue bank with 11,000 annotated samples, a 31,000-patient research database.

The Moon Shots Program adds access to important new research infrastructure. For example, the MDS/AML moon shot will benefit from new platforms in genomics and bioinformatics that allow large-scale molecular analysis to characterize hundreds of patient samples at various stages of the disease.

MOON SHOT GOALS

- Develop new molecularly targeted therapies in the first five years.
- Identify the causes of resistance to treatment and translate knowledge into significant cure rates for older AML and MDS patients within five years.
- Generate the first mouse model of MDS to test hypotheses.

The top four projects are:

- Cure MDS by developing models to identify "low-risk" patients who actually have poor prognosis and early; identify new drug targets for low- and high-risk disease; unraveling mechanisms of resistance to existing drugs and the drivers of transformation to AML.

-more-

- Cure AML by identifying new therapeutic targets, optimizing current treatments and finding new ways to avoid relapse.
- Focus on immunotherapy by developing non-toxic stem cell transplant approaches, improving donor-matched and cord blood transplants and advancing new forms of immunotherapy.
- Uncover the causes of MDS and AML with large-scale population studies and focus on the mechanisms that drive MDS linked to prior cancer treatment.

THE AIM

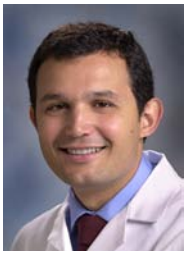
The MDS/AML moon shot will more efficiently apply existing technology and knowledge to improve survival in the near term while conducting break-through studies to understand and ultimately cure these diseases. The development continuum of MDS and AML also provides an ideal opportunity to study the evolution of cancer from early stage to advanced disease.

.

THE MDS/AML MOON SHOT TEAM

This moon shot gathers a diverse team of experts from across the institution, including leaders in oncology, immunology, genomics, stem cell transplantation, pathology, basic science, biostatistics, epidemiology, translational research and drug development.

Moon shot leaders



Guillermo Garcia-Manero, M.D., Ph.D. – Professor and chief of the MDS Section in MD Anderson's Department of Leukemia. Garcia-Manero is a world leader in translational research, clinical investigation and treatment of MDS and AML. He also conducts extensive research developing prognostic models for these cancers, including one to identify apparently low-risk MDS patients who actually have poor prognosis.

Hagop Kantarjian, M.D., professor and chair of MD Anderson's Department of Leukemia, the world's largest program. The department's internationally recognized expertise in clinical translation allows it to offer the broadest array of clinical trials for new therapies. Most patients are on treatment protocols that either include standard of care plus an investigational drug or combinations of investigational new drugs. Kantarjian also is associate vice president for global academic programs.

###