MD Anderson's Moon Shots Program: Transforming Cancer Prevention and Care



Making Cancer History®

Ronald A. DePinho, M.D. President

Our Mission

To eliminate cancer in Texas, the nation and the world through outstanding programs that integrate patient care, research and prevention, and through education for undergraduate and graduate students, trainees, professionals, employees and the public.

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21,000 People, One Goal

Our singular focus: Making Cancer History

Our organizing principle: Serve the patient

Our culture: Collaboration

Our strategy: Research-driven patient care

Serve the Patient

MD Anderson team

- 21,000 employees, including nearly 1,000 physicians and 3,000 nurses
- More than 1,000 volunteers
- 1,700 faculty across
 66 departments

Our size and volume:

- Enables multidisciplinary care
- · Builds experience in common and rare cancers
- · Provides unique options for our patients: technology and trials

Patient-Focused Growth

Clinical and Translational Investments

- Alkek Hospital Expansion: 24 floors, 645 inpatient beds
- 16 million gross square feet
 in the Texas Medical Center
- · Major new buildings
 - Pavilion for state-of-the-art surgery and interventional radiology
 - Zayed Building for Personalized
 Cancer Care



Total patients served

127,000 (Houston)114,000 (Network)

Our Research Excellence



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Unprecedented Opportunity in Cancer Medicine

- Deep knowledge of biology
- Disruptive technologies
- Critical mass & global reach





New paradigms are needed to ensure discoveries become clinical endpoints Image: Clinical endpoints <tr





The Moon Shots Program

Goal-oriented multi-disciplinary effort harnessing available knowledge and new disruptive technologies to dramatically reduce cancer mortality through prevention, early detection and curative treatments

The Moon Shots Program

- Flagship projects underway designed to impact cancer mortality
- Projects span prevention, early detection and treatment areas
- Platforms enable execution through expert professionals
- Collaborations expanding and funding growing: ~\$223M

The Moon Shots Program

Currently funded moon shots

- Leukemia MDS/AML
- Leukemia CLL
- Prostate cancer
- Melanoma
- Lung cancer
- Breast and Ovarian cancer

Collectively ~50% of cancer deaths

Moon Shot Platforms

Cancer Prevention and

 APOLLO & Big Data Clinical Genomics Research Genomics Proteomics

Immunotherapy

 Institute for Personalized Cancer Therapy

Control

- Pilot moon shots
- Glioblastoma Pancreatic cancer
- HPV-associated cancers
- Colorectal cancer
- B-cell Lymphoma
- Multiple Myeloma

- Institute of Applied
 Cancer Sciences
 - Oncology Research for Biologics & Immunotherapy Translation (ORBIT)

 - Applied Cellular Therapy
 - Center for Co-Clinical Trials

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Cancer Prevention and Control Platform

Up to 50% of cancers can be prevented



Policy, education and evidence-based service delivery can make a measurable and lasting difference in the community, especially among the underserved

Melanoma Moon Shot Team and Cancer Control Platform

- Excessive UV exposure during childhood drives a significant increase in melanoma
- Collaborative efforts have led to positive outcomes via education and policy
 - Early childhood education (K-12) programming
 - Preschool sun protection curriculum
 - Tanning bed legislation passed in Texas and nine other states
 Currently proposed in 15 other states

Immunotherapy: Paradigm Shift



Immunotherapy combos: Game-changer 100 1 Yr OS 94% 2 Yr OS 88% 90 80 ٦. 2 Yr OS 79% 70 1 Yr OS 85% Survival (%) 60 50 1 Yr OS 57% 2 Yr OS 50% 40 30 Nivo 0.3 mg/kg + IPI 3 mg/kg (n=14) Nivo 1 mg/kg + IPI 3 mg/kg (n=17) Nivo 3 mg/kg + IPI 1 mg/kg (n=16) Nivo 3 mg/kg (n=6) Concurrent Cohorts 1 ~ (n=63) 20 2 10

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Immunotherapy Platform

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· Director, James Allison

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- Blends world-class expertise and leading technology
- Enables researchers to take the newest immune-modifying drugs, test them in both preclinical and clinical settings, and apply deep analyses for mechanistic insight
- · Facilitates clinical trials across diverse tumor types
- Aggregates clinical and research data (APOLLO & Big Data)



Ovarian Cancer Moon Shot

- No improvement in survival for decades
- · Survival correlates with complete surgical resection of disease
- Pioneered the Anderson algorithm for personalized surgery
 - Less-invasive laparoscopy: two oncologists independently assess and objectively score extent of disease
 Proceed to surgery or chemotherapy to reduce tumor burden
 - before surgery Result: complete removal in ~90%, compared to 20% Focus: dissemination of practice-changing approach









Lung Cancer Moon Shot

Integrated plan to significantly advance prevention, early detection, and molecularly-guided treatment of lung cancer











Institute for Personalized Cancer Therapy

- Created to support preclinical research and clinical trials in which a patient's tumor biopsy is assayed for abnormal genes and gene products to select therapy with agents targeting the product of those particular abnormal genes
- This integrated research and clinical trials program is aimed at implementing personalized cancer therapy and improving patient outcomes

Big Data Platform

- Integrates systems to improve patient outcomes, such that every patient contributes to and potentially benefits from research
- Dismantles silos that impede research-driven patient care, gathers data related to questions across tumor types, increases efficient use of data, focuses on a foundation built to secure and protect data and maintains historical accuracy of legacy data



APOLLO Platform: Adaptive Patient-Oriented Longitudinal Learning and Optimization

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3 Core Functionalities of MD Anderson OEA™

- Dynamic patient summary that assimilates complete
 patient data
- Evidence-based treatment options with supporting evidence, including clinical trial options
- Care pathway advisory to better manage patient on therapy for optimal outcome





Our Record in Translating Discoveries Into Effective Medicines



Conceptual:

Knowledge gap, limited technology and models
 Lack of clear line-of-sight for clinical development

- Organizational:
 - Basic biologists, clinical disease experts, and professional drug developers are physically separated
 Poor integration and hand-off between stages



Institute for Applied Cancer Science: **Drug Development Platform**

Rapid discovery of new drugs that are clinically relevant: **small molecules & biotherapeutics** Operational since January 2012: ~75 employees led by >200 years industry experience Rigorous evaluation of targets & drugs, generate real drugs, focus on defining the clinical path hypothesis with biomarker-driven execution













Developing a Sustainable Model for Mission Support ⁸⁹ ⁹⁰ ¹ ¹ ² ³ ¹ ¹ ¹ ² ³ ¹ ¹

Newco's

- Achieved via Strategic Alliances with Big Pharma, risk-sharing agreements with smaller biotechs, Asset Development Corporations for individual drug candidates, or NewCo formation.
- All arrangements are guided by our conflict of interest rules to preserve research integrity and the safety of our patients.



Optimize Strategic Alliances to 'Move the Clinical Needle'

- Interactions with Industry have been limited to a specific field and heavily regulated by the sponsor
- Innovation alternative includes Investigator-Initiated Studies with limited (or zero) industry support; even in those cases, the key drugs are difficult to obtain, particularly if they are in pivotal clinical trials
- We can be far more successful if we work collaboratively, with meaningful input from both parties toward a common goal
- Solution: Pioneer novel interaction models where we:
- · Gain access to all the truly impactful drugs, with few restrictions
- Are able to guide the course of collaboration, and stay involved throughout
- · Enable all of that with substantial funding from the pharma partner

University of Texas MD Anderson Cancer C





Strategic A-to-Z Alliance with Amgen Harnessing Proteomics, Novel Drug Platform









Fulfilling a Solemn Responsibility To Our Patients, Future Generations

"Knowing is not enough; we must apply. Willing is not enough; we must do."



Johann Wolfgang von Goethe

