This event is made possible with generous support from:

- Bristol-Myers Squibb
- GSK
- Merck
- Immunotherapy Foundation
- Genentech
- Lilly Oncology
- Regeneron
- Sanofi
- Genzyme
- Novartis
- Pfizer
Our Educational Partners

- Addario Lung Cancer Foundation
- American Cancer Society
- Baylor College of Medicine
- But Doctor I Hate Pink (Ann Silberman)
- Cancer Support Community
- CancerCare
- Colorectal Cancer Alliance
- Fight Colorectal Cancer
- FORCE
- Houston Methodist Cancer Center
- Imerman Angels

Thank you to those who helped promote the summit

- Leukemia & Lymphoma Society
- LUNGevity Foundation
- The Learning Center at MD Anderson Cancer Center
- Let Life Happen (Barbara Jacoby)
- MD Anderson Cancer Center
- Patient Empowerment Network
- The Rose
- Sisters Network
- SHARE
- Susan G. Komen
- Us TOO
<table>
<thead>
<tr>
<th>Speakers</th>
<th>Patient Experts</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Scientific Experts</strong></td>
<td><strong>Patient Experts</strong></td>
</tr>
<tr>
<td>Adi Diab, M.D.</td>
<td>K.C. Dill</td>
</tr>
<tr>
<td>MD Anderson Cancer Center</td>
<td>Lung Cancer</td>
</tr>
<tr>
<td>Renata Ferrarotto, M.D.</td>
<td>Robert Fitzgerald</td>
</tr>
<tr>
<td>MD Anderson Cancer Center</td>
<td>Melanoma</td>
</tr>
<tr>
<td>Andrew Sikora, M.D., Ph.D.</td>
<td>Rick Frantz</td>
</tr>
<tr>
<td>Baylor College of Medicine</td>
<td>Kidney Cancer</td>
</tr>
<tr>
<td>Cassian Yee, M.D.</td>
<td>Kathy Vecchio</td>
</tr>
<tr>
<td>MD Anderson Cancer Center</td>
<td>Non-Hodgkin Lymphoma</td>
</tr>
<tr>
<td>Jun Zhang, M.D.</td>
<td></td>
</tr>
<tr>
<td>Time</td>
<td>Event</td>
</tr>
<tr>
<td>-------</td>
<td>----------------------------------------------------------------------</td>
</tr>
<tr>
<td>9:00 am</td>
<td>Registration and networking</td>
</tr>
<tr>
<td>10:00 am</td>
<td>Program commences</td>
</tr>
<tr>
<td>10:15 am</td>
<td>WELCOME</td>
</tr>
<tr>
<td></td>
<td>Brian Brewer</td>
</tr>
<tr>
<td>10:15 am</td>
<td>HEAR FROM THE EXPERTS</td>
</tr>
<tr>
<td></td>
<td>Immunotherapy Basics</td>
</tr>
<tr>
<td></td>
<td>Andrew Sikora, M.D., Ph.D.</td>
</tr>
<tr>
<td>10:45 am</td>
<td>Panel: Research Updates</td>
</tr>
<tr>
<td></td>
<td>Moderator</td>
</tr>
<tr>
<td></td>
<td>Andrew Sikora, M.D., Ph.D.</td>
</tr>
<tr>
<td></td>
<td>Panelists</td>
</tr>
<tr>
<td></td>
<td>Renata Ferrarotto, M.D.</td>
</tr>
<tr>
<td></td>
<td>Cassian Yee, M.D.</td>
</tr>
<tr>
<td></td>
<td>Jun Zhang, M.D.</td>
</tr>
<tr>
<td>11:30 am</td>
<td>PATIENT PERSPECTIVE</td>
</tr>
<tr>
<td></td>
<td>A message from K.C. Dill, lung cancer veteran</td>
</tr>
<tr>
<td>12:00 pm</td>
<td>Lunch and networking</td>
</tr>
<tr>
<td>1:00 pm</td>
<td>LEARN ABOUT CLINICAL TRIALS</td>
</tr>
<tr>
<td></td>
<td>Brian Brewer</td>
</tr>
<tr>
<td>1:15 pm</td>
<td>IMMUNOTHERAPY PATIENT PANEL</td>
</tr>
<tr>
<td></td>
<td>Moderator</td>
</tr>
<tr>
<td></td>
<td>Brian Brewer</td>
</tr>
<tr>
<td></td>
<td>Panelists</td>
</tr>
<tr>
<td></td>
<td>Robert Fitzgerald</td>
</tr>
<tr>
<td></td>
<td>Rick Frantz</td>
</tr>
<tr>
<td></td>
<td>Kathy Vecchio</td>
</tr>
<tr>
<td>2:00 pm</td>
<td>TRANSITION BREAK</td>
</tr>
<tr>
<td>2:15 pm</td>
<td>BREAKOUT SESSIONS</td>
</tr>
<tr>
<td></td>
<td>Your choice of a deeper dive Q&amp;A with our experts</td>
</tr>
<tr>
<td></td>
<td>General Immunotherapy</td>
</tr>
<tr>
<td></td>
<td>Andrew Sikora, M.D., Ph.D.</td>
</tr>
<tr>
<td></td>
<td>Head &amp; Neck Cancer</td>
</tr>
<tr>
<td></td>
<td>Renata Ferrarotto, M.D.</td>
</tr>
<tr>
<td></td>
<td>Melanoma</td>
</tr>
<tr>
<td></td>
<td>Cassian Yee, M.D.</td>
</tr>
<tr>
<td></td>
<td>Genitourinary Cancer</td>
</tr>
<tr>
<td></td>
<td>Jun Zhang, M.D.</td>
</tr>
<tr>
<td>3:15 pm</td>
<td>Program closes</td>
</tr>
<tr>
<td>9:00 am - 4:00 pm</td>
<td>CLINICAL TRIAL NAVIGATOR APPOINTMENTS</td>
</tr>
<tr>
<td></td>
<td>Appointments are available all day. If you didn’t pre-register, but you</td>
</tr>
<tr>
<td></td>
<td>are interested in scheduling an appointment, please visit the</td>
</tr>
<tr>
<td></td>
<td>Clinical Trial Navigator desk for more information.</td>
</tr>
</tbody>
</table>
You will receive two emails after the summit:

1. **A survey** to share your feedback on the summit as well as insights into future programming.

2. **Information** from the summit day, including this presentation and instructions on how to use our [Clinical Trial Finder service](#).
Andrew Sikora, M.D., Ph.D.

Caroline Weiss-Law Translational Research Scholar
Co-director of the Head and Neck Cancer Program at Baylor College of Medicine
Origin & Revival of Immunotherapy

1890s: William B. Coley
1900s: Paul Ehrlich
1960s: Lloyd J. Old
Immunotherapy is “hand to hand combat”

Photo Credit: Rita Serda, PhD; National Cancer Institute “Cancer Close Up” winner 2016
How many immune cells are in the human body?
How many immune cells are in the human body?

$$10^{12} \quad > \quad 10^{11}$$
Immunotherapy: Potential for Cure?

Why MORE RESEARCH is needed

Other approaches

Survival vs. Time

IT

65 YEARS IN IMMUNOTHERAPY
CANCER RESEARCH INSTITUTE

IMMUNOTHERAPY PATIENT SUMMIT
The Immune System At a Glance

**Nose**
- Hairs and mucus trap foreign particles and prevent them from entering the body.

**Thymus**
- Small organ located just behind the breastbone where T cells mature (the "T" is for thymus).

**Bone marrow**
- Tissue in the center of bones that is responsible for making blood cells, including white blood cells.

**White blood cells**
- White blood cells—including macrophages, dendritic cells, and lymphocytes—are the cellular actors of immunity.

**Tonsils**
- Structures at the back of the throat that sample bacteria and viruses that enter the body through the mouth or nose.

**Lymph nodes**
- Small, bean-shaped structures located throughout the body that filter lymph fluid, where immune cells are alerted to the presence of pathogens or cancer.

**Spleen**
- Fist-sized organ located in the upper-left part of the abdomen, containing white blood cells that fight infection and cancer.

**Lymphatic vessels**
- Thin-walled tubes that collect and transport lymph fluid throughout the body.
The Cells of the Immune System

- Dendritic Cell
- Monocyte
- Neutrophil
- B Cell
- Natural Killer Cell
- Macrophage
- T Cell
Immunotherapy approaches

**Stimulating** existing immune cells (e.g. vaccines)

**Delivering more** immune cells into the patient (e.g. adoptive cell therapy)

**Inactivating** cancer’s immune defenses (e.g. checkpoint inhibitors)
Adaptive Immune Responses Against Cancer

Cancer Cell
(being engulfed)

Antigen-Presenting Cell
(e.g., Dendritic Cell)
Adaptive Immune Responses Against Cancer

Antigen-Presenting Cell (e.g., Dendritic Cell)

Tumor Antigens
Adaptive Immune Responses Against Cancer

Antigen-Presenting Cell (e.g., Dendritic Cell)

Tumor Antigen (bound by MHC1)

T Cell Receptor (TCR)
Adaptive Immune Responses Against Cancer

ACTIVATED "KILLER" T CELL

Antigen-Presenting Cell (e.g., Dendritic Cell)
Adaptive Immune Responses Against Cancer

Cancer Cell

Activated “killer” T Cell
Adaptive Immune Responses Against Cancer

CANCER CELL ELIMINATED!
Immune Checkpoints Can Suppress Immune Responses

Activated “killer” T Cell

Cancer Cell

PDL1 - PD1
Immune Checkpoints Can Suppress Immune Responses

Normally, PDL1-PD1 leads to T cell “exhaustion”
Checkpoint Immunotherapy Can Promote Anti-Cancer Activity

Cancer Cell

Activated “killer” T Cell

PD-1/PD-L1 Checkpoint Inhibitors
Activated "killer" T Cell Cancer Cell

PD-1/PD-L1 Pathway Blocked!

Checkpoint Immunotherapy Can Promote Anti-Cancer Activity
Checkpoint Immunotherapy Can Promote Anti-Cancer Activity

Cancer Cell

Activated “killer” T Cell

CANCER CELL ELIMINATED!
Adoptive T Cell Immunotherapy

1. Isolation
2. Activation
3. Expansion
4. Re-infusion
Adoptive T Cells In Action (Against Melanoma)
Equip T cells with new, cancer-targeting receptors
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)

CARs enable MHC-independent targeting & killing!
CARs enable MHC-independent targeting & killing!
• Viruses can alter our cells’ DNA, by inserting their own genetic material
• Impaired defenses make tumor cells more susceptible to infection
AFTER INJECTION:

1) Viruses cause tumor cells to “burst” & release antigens
2) Immune cells uptake & present tumor antigens
3) Stimulates adaptive, and potentially systemic, immune responses
Reprogramming Oncolytic Viruses To Enhance Anti-Tumor Activity

(+) INSERT immune-stimulating genes

(—) REMOVE Disease-causing genes (selective targeting of tumors)
Cancer Vaccines

Tumor Antigens
(provided by vaccine)
Dendritic cell
Cancer Vaccines

Dendritic cell

T cell
Cancer Vaccines

ACTIVATED
“KILLER” T CELL

Dendritic cell
Vaccine-Induced Elimination of Cancer Cells

Cancer Cell

Activated “killer” T Cell
Vaccine-Induced Elimination of Cancer Cells

Cancer Cell

Activated “killer” T Cell
Challenges in Cancer Immunotherapy

• Discovering **new immune pathways, targets, and strategies** to increase the number of immunotherapy approaches available

• Identifying and validating new biomarkers to help doctors **predict** which patients will **respond** to immunotherapies

• Determining the best way to **combine** immunotherapies with each other and with conventional treatments to **benefit** more patients

• Learning how to **reduce side effects** of immunotherapy without losing benefit
Panel Discussion

LATEST RESEARCH UPDATES
<table>
<thead>
<tr>
<th>Moderator</th>
<th>Panel</th>
</tr>
</thead>
<tbody>
<tr>
<td>Andrew Sikora, M.D., Ph.D.</td>
<td>Renata Ferrarotto, M.D.</td>
</tr>
<tr>
<td></td>
<td>Head and Neck Cancers</td>
</tr>
<tr>
<td>Cassian Yee, M.D.</td>
<td>Melanoma</td>
</tr>
<tr>
<td>Jun Zhang, M.D.</td>
<td>Lung Cancer and Non-Prostate Genitourinary Cancers</td>
</tr>
</tbody>
</table>
K.C. Dill
Surviving Lung Cancer

PATIENT PERSPECTIVE
Lunch and Networking

Ballroom
Brian Brewer
Cancer Research Institute

LEARN ABOUT CLINICAL TRIALS
What Are Clinical Trials?

- Research studies that involve people
- Designed to answer specific questions about new and existing treatments
- Aim to improve treatments and the quality of life for people with disease
Getting from Discovery to Approval

- **Pre-Discovery**: 3 - 6 years, ~5,000-10,000 compounds
- **Drug Discovery**: 250
- **Preclinical**: 5
- **Clinical Trials**: Phase 1 (20-100), Phase 2 (100-500), Phase 3 (1,000-5,000), 6 - 7 years
- **FDA Review**: 0.5 - 2 years
- **Scale-Up to Mfg.**: INDEFINITE
- **Post-Marketing Surveillance**: INDEFINITE

Source: AppliedClinicalTrials.com
What Are Clinical Trial Phases?

**Phase 1**
- Is the treatment safe?
- Purpose:
  - First study in humans
  - Find best dose, delivery method, and schedule
  - Monitor for side effects
  - Determine safety
- Number of people: 20-100

**Phase 2**
- Does it work?
- Purpose:
  - Look for effect on specific type(s) of cancer
  - Continue monitoring for side effects and safety
- Number of people: 100-500

**Phase 3**
- Does it work better?
- Purpose:
  - Compare new treatment (or new use of a treatment) with current standard treatment
  - Determine risk vs. benefit
- Number of people: 1,000-5k+
## Pros and Cons of Clinical Trials

<table>
<thead>
<tr>
<th>Potential Advantages</th>
<th>Potential Disadvantages</th>
</tr>
</thead>
<tbody>
<tr>
<td>Access to best possible care</td>
<td>Unknown side effects or risks</td>
</tr>
<tr>
<td>Receiving new drugs before they’re widely available</td>
<td>Unknown benefits—drugs may not work as intended</td>
</tr>
<tr>
<td>Close monitoring by medical team</td>
<td>Not all patients may benefit</td>
</tr>
<tr>
<td>Chance to play active role in healthcare and research</td>
<td>Frequent tests and clinic visits</td>
</tr>
<tr>
<td>Help future generations</td>
<td>Possible need to travel to trial sites</td>
</tr>
</tbody>
</table>

Questions to Ask Before Volunteering

• Why is this trial being done?
• Why is it believed that the treatment being studied may be better than the standard treatment?
• What are my other options (standard treatments, other trials)?
• How did patients do in any previous studies of this treatment?
• How will the doctor know if treatment is working?
• How long will the trial last?

Questions to Ask Before Volunteering

• Can I continue to receive this treatment after the trial ends?
• What kinds of procedures or tests are involved?
• What impact will the trial have on my daily life?
• Will I have to travel for treatment? Will I be compensated?
• How often will I need to travel to receive treatment?
• Will I be hospitalized as part of the trial?
• What costs (if any) will be my responsibility to pay?

Getting into a Clinical Trial Isn't Always a Given

Trials are designed to ask specific questions, and must adhere strictly to entry criteria to ensure data is accurate and meaningful.

This also helps ensure patients who could be made worse by treatment are not exposed to the risk.

Common criteria include:
- cancer type or stage
- treatment history
- genetic factors
- age
- medical history
- current health status
I might only get placebo ("sugar pill") instead of treatment.

Placebos are rarely used and never given in the absence of some form of treatment.

Clinical Trials: Myth versus Fact

**MYTH**

Trials are only for people who have run out of treatment options (a “last resort”).

**FACT**

Clinical trials are designed for people with cancer of all types and stages.

*Patient Resource*, “Understanding Clinical Trials: A Guide for Patients and Their Families”
Clinical Trials: Myth versus Fact

**MYTH**
I need to travel to a large hospital or cancer center to participate in a clinical trial.

**FACT**
Trials take place at local hospitals, cancer centers, and doctors’ offices in all parts of the country, in both urban and rural areas.

Clinical Trials: Myth versus Fact

**MYTH**
My health insurance doesn’t cover the cost of care in a clinical trial.

**FACT**
Doctor visits, hospital stays, and certain testing procedures may be covered by insurance. Research costs are typically covered by the trial sponsor.

Clinical Trials: Myth versus Fact

**MYTH**

Signing a consent form “locks” me into staying in a trial.

**FACT**

Fact: You are free to change your mind for any reason about participating in a trial anytime before or during a trial.

Clinical Trials: Myth versus Fact

I will be made to feel like a “guinea pig” experiment.

Fact: The overwhelming majority of trial participants say they were treated with dignity and respect, and report having had a positive experience in a trial.

Clinical Trials: Myth versus Fact

**MYTH**
Clinical trials aren't safe.

**FACT**
Fact: Safeguards including an Institutional Review Board, Data and Safety Monitoring Board, and an ongoing informed consent process ensure patients’ rights and safety are protected.

*Patient Resource*, “Understanding Clinical Trials: A Guide for Patients and Their Families”
A Word About Informed Consent

Informed consent = having all the facts before and during a trial

- Study purpose
- Length of time of the study
- Predictable risks
- Possible benefits
- Expectations
- Patient’s rights

- Treatment alternatives
- Patient health monitoring
- Safeguards in place
- How to withdraw from study

Be bold in asking for details. It’s YOUR treatment plan.
How Can I Find a Clinical Trial?

• Ask your doctor
• Ask another doctor if necessary...
• Contact a patient advocacy organization
  – Seek assistance from a clinical trial navigator, if offered
  – CRI Clinical Trial Finder: 1 (855) 216-0127
• Search online
  – https://www.cancerresearch.org/patients/clinical-trials
  – https://clinicaltrials.gov/
Panel Discussion

Immunotherapy Patient Panel
<table>
<thead>
<tr>
<th>Moderator</th>
<th>Panel</th>
</tr>
</thead>
<tbody>
<tr>
<td>Brian Brewer</td>
<td>Robert Fitzgerald</td>
</tr>
<tr>
<td></td>
<td>Melanoma</td>
</tr>
<tr>
<td></td>
<td>Rick Frantz</td>
</tr>
<tr>
<td></td>
<td>Kidney Cancer</td>
</tr>
<tr>
<td></td>
<td>Dixie Frantz</td>
</tr>
<tr>
<td></td>
<td>Kidney Cancer</td>
</tr>
<tr>
<td></td>
<td>Kathy Vecchio</td>
</tr>
<tr>
<td></td>
<td>Non-Hodgkin Lymphoma</td>
</tr>
</tbody>
</table>
BREAKOUT SESSIONS
<table>
<thead>
<tr>
<th>Topic</th>
<th>Room</th>
</tr>
</thead>
<tbody>
<tr>
<td>General Immunotherapy</td>
<td>Ballroom</td>
</tr>
<tr>
<td>Renata Ferrarotto, M.D.</td>
<td></td>
</tr>
<tr>
<td>Genitourinary and Lung Cancers</td>
<td>Room 5</td>
</tr>
<tr>
<td>Jun Zhang, M.D.</td>
<td></td>
</tr>
<tr>
<td>Melanoma</td>
<td>CPB Telehealth</td>
</tr>
<tr>
<td>Adi Diab, M.D.</td>
<td></td>
</tr>
</tbody>
</table>
This event is made possible with generous support from:

- Bristol-Myers Squibb
- gsk
- MERCK
- Immunotherapy Foundation
- Genentech
- Lilly Oncology
- REGENERON
- SANOFI GENZYME
- CANCER RESEARCH INSTITUTE
- IMMUNOTHERAPY PATIENT SUMMIT
- Novartis
- Pfizer
Our Educational Partners

Thank you to those who helped promote the summit

- Addario Lung Cancer Foundation
- American Cancer Society
- Baylor College of Medicine
- But Doctor I Hate Pink (Ann Silberman)
- Cancer Support Community
- CancerCare
- Colorectal Cancer Alliance
- Fight Colorectal Cancer
- FORCE
- Houston Methodist Cancer Center
- Imerman Angels

- Leukemia & Lymphoma Society
- LUNGevity Foundation
- The Learning Center at MD Anderson Cancer Center
- Let Life Happen (Barbara Jacoby)
- MD Anderson Cancer Center
- Patient Empowerment Network
- The Rose
- Sisters Network
- SHARE
- Susan G. Komen
- Us TOO
Thank You!

You will receive two emails after the summit:

1. **A survey** to share your feedback on the summit as well as insights into future programming.

2. **Information** from the Summit day, including this presentation and instructions on how to use our [Clinical Trial Finder service](#).