CANCER RESEARCH INSTITUTE
IMMUNOTHERAPY PATIENT SUMMIT

Baltimore  November 16, 2019
WELCOME

Brian Brewer
Cancer Research Institute
<table>
<thead>
<tr>
<th>Scientific Experts</th>
<th>Patient Experts</th>
</tr>
</thead>
<tbody>
<tr>
<td>Marijo Bilusic, M.D., Ph.D.</td>
<td>Vanessa Brandon</td>
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<td>National Cancer Institute</td>
<td>Colorectal cancer</td>
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<td><strong>Donna Lynch</strong></td>
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<td>Johns Hopkins Kimmel Cancer Center</td>
<td>Diffuse large B-cell (non-Hodgkin) lymphoma</td>
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<td><strong>Ranee Mehra, M.D.</strong></td>
<td><strong>John Ryan</strong></td>
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<td>University of Maryland Medical Center</td>
<td>Non-small cell lung cancer</td>
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<td><strong>Suzanne L. Topalian, M.D.</strong></td>
<td><strong>Adrienne Skinner</strong></td>
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<td>Johns Hopkins Kimmel Cancer Center</td>
<td>Ampullary cancer</td>
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This event is made possible with generous support from:

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

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- Imerman Angels
- HopeWell Cancer Care
- Johns Hopkins Sidney Kimmel Cancer Center
- Leukemia & Lymphoma Society
- Ludwig Cancer Research
- LUNGevity
- Melanoma Research Alliance
- Melanoma Research Foundation
- National Ovarian Cancer Coalition – Baltimore
- Nueva Vida
- Pancreatic Cancer Action Network
- Patient Empowerment Network
- SHARE
- Us TOO
- Ulman Foundation
- University of Maryland Medical Center
- Young Survival Coalition
## Summit Program

<table>
<thead>
<tr>
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<th>Time</th>
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<tbody>
<tr>
<td>Morning Session</td>
<td>10:00 AM – 12:00 PM</td>
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<tr>
<td>Lunch</td>
<td>12:00 PM – 1:00 PM</td>
</tr>
<tr>
<td>Afternoon Session</td>
<td>1:00 PM – 2:15 PM</td>
</tr>
<tr>
<td>Breakout Sessions</td>
<td>2:15 PM – 3:15 PM</td>
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**Clinical Trial Navigator Appointments** are available from 9:00 AM to 4:00 PM. Please stop by the check-in desk near registration to learn more.
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](#).
SAVE MORE LIVES
by fueling the discovery
and development of
powerful immunotherapies
for all types of cancer.

Sharon Belvin
Melanoma Survivor & Mom
FUNDED 3,300 scientists worldwide
INVESTED $420+ million
TRUSTED Platinum, A+ charity
Immunotherapy 101

Elizabeth M. Jaffee, M.D.
Deputy Director, The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins
Professor of Oncology
Origin & Revival of Immunotherapy

1890s: William B. Coley
1900s: Paul Ehrlich
1960s: Lloyd J. Old
Immunotherapy: A Potential Cure?

- **Standard therapy**
  - Pts take longer to progress, but succumb at same rate

- **Immunotherapy**
  - Increased survival

Room for improvement

<table>
<thead>
<tr>
<th>Percent alive</th>
<th>years</th>
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<tbody>
<tr>
<td>100</td>
<td>1</td>
</tr>
<tr>
<td>100</td>
<td>2</td>
</tr>
<tr>
<td>0</td>
<td>3</td>
</tr>
<tr>
<td>0</td>
<td>4</td>
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</tbody>
</table>
The Immune System at a Glance:
Our Natural Defense System

- **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: The “Soldiers” in our Army
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)
Adaptive Immune Responses Against Cancer

T Cell Receptor (TCR)

Tumor Antigen (bound by MHC1)

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

T Cell Receptor (TCR)
Adaptive Immune Responses Against Cancer

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

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

ACTIVATED “KILLER” T CELL
Adaptive Immune Responses Against Cancer

Cancer Cell

Activated “killer” T Cell
Adaptive Immune Responses Against Cancer
Adaptive Immune Responses Against Cancer

CANCER CELL ELIMINATED!

Activated “killer” T Cell
Activated “killer” T Cell

Cancer Cell

PDL1

PD1

Immune Checkpoints Can Suppress Immune Responses
Immune Checkpoints Can Suppress Immune Responses

Cancer Cell

Activated “killer” T Cell

PDL1-PD1
Immune Checkpoints Can Suppress Immune Responses

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

Activated "killer" T Cell

PD-1/PD-L1 Checkpoint Inhibitors
Checkpoint Immunotherapy Can Promote Anti-Cancer Activity
Checkpoint Immunotherapy Can Promote Anti-Cancer Activity

Cancer Cell

Activated “killer” T Cell
Activated “killer” T Cell Can Promote Anti-Cancer Activity

PD-1/PD-L1 Pathway Blocked!
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 TCR
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)

Cancer Cell

CAR T Cell

CARs enable MHC-independent targeting & killing!
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)

CARs enable MHC-independent targeting & killing!
CAR T Cell Immunotherapy (Chimeric Antigen Receptor)

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

Oncolytic Virus Immunotherapy
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)
Cancer Vaccines

Dendritic cell

Tumor Antigens (provided by vaccine)
Cancer Vaccines

Dendritic cell
Cancer Vaccines

Dendritic cell

T cell
Vaccine-Induced Elimination of Cancer Cells

Cancer Cell

Activated “killer” T Cell
Activated “killer” T Cell

Vaccine-Induced Elimination of Cancer Cells
Personalized Neoantigen Vaccine Trial
Challenges in Cancer Immunotherapy

- Discovering and validating new biomarkers to help doctors predict which patients will respond to which immunotherapies

- Determining the best way to combine immunotherapies with each other as well other treatments to extend immunotherapy's benefits for more patients

- Learning how to decouple side effects of immunotherapy from benefit
Why have most responses been modest and why are some cancers refractory to immunotherapy?

1. Cancers upregulate molecules to turn off immune cells
Why have most responses been modest and why are some cancers refractory to immunotherapy?

1. Cancers upregulate molecules to turn off immune cells

2. Cancers secrete chemicals to turn off the immune system
Why have most responses been modest and why are some cancers refractory to immunotherapy?

1. Cancers upregulate molecules to turn off immune cells
2. Cancers secrete chemicals to turn off the immune system
3. Cancers recruit suppressive cells to inactivate/block the immune response
Panel Discussion

LATEST RESEARCH UPDATES

Panelist
Marijo Bilusic, M.D., Ph.D.
Genitourinary cancer

Panelist
Ranee Mehra, M.D.
Lung cancer

Panelist
Suzanne L. Topalian, M.D.
Melanoma

Moderator
Elizabeth M. Jaffee, M.D.
Pancreatic cancer
Immunotherapy Patient Perspective

Adrienne Skinner
Ampullary Cancer Veteran

#CRIsummit
Adrienne’s Immunotherapy Experience

Adrienne’s Guidelines

• **Clinical trials’ success depends on specific characteristics for each trial:** Find out what those are and be prepared to answer questions about your ability to meet them

• **Cancer treatment is a journey:** Make sure you are exploring all roads

• **Become educated:** However, trust but verify your sources

• **Remember there are thousands of people who are working to help you:** Your experience is important in ways you may never know. Help the process of improving results for all

• **Persistence and positivity makes a difference**

• **Take control where you can**

• **Marshall your support team**
Adrienne’s Immunotherapy Experience

Cancer makes you feel like your body is out of control.

Choosing when my hair would be gone felt better than waiting for it.

Taking control of my hair loss in 2013.
Adrienne’s Immunotherapy Experience

No one really knew I was sick unless I told them. How you behave is what people read. Make-up, wigs and false eyelashes hid the physical impact for me.

This is my ‘fancy girl’ wig. She came to work with me.
Adrienne’s Immunotherapy Experience

Dr. Luis Diaz and Dr. Yung Le, my heros!

These are the amazing doctors who led the clinical trial for the immunotherapy solution that saved my life.
Adrienne’s Immunotherapy Experience

Support from them was, and is, crucial. And for them, knowing there is a potential solution should they get cancer is a relief (three of them have Lynch Syndrome, too).

My four daughters
Adrienne’s Immunotherapy Experience

Support from family and friends is essential. Be open about what you need.

So lucky to have Joe in my life!
Adrienne’s Immunotherapy Experience

What a joy to be here for my first grandchild.

My newest team support member!
Lunch and Networking
North and South Dining Terrace
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
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>
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<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>
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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.

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

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.

My health insurance doesn't cover the cost of care in a clinical trial.

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.

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.

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/
# Immunotherapy Patient Panel

<table>
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<tr>
<th>Moderator</th>
<th>Panel</th>
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| Brian Brewer       | Vanessa Brandon
|                    | Colorectal cancer                                                  |
|                    | Donna Lynch
|                    | Diffuse large B-cell (non-Hodgkin) lymphoma                       |
|                    | John Ryan
<p>|                    | Non-small cell lung cancer                                         |</p>
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<td>General Immunotherapy</td>
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<tr>
<td>Elizabeth M. Jaffee, M.D.</td>
</tr>
<tr>
<td>Melanoma</td>
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<tr>
<td>Megan D. Schollenberger, MSN, CRNP</td>
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