

A woman with curly hair, wearing a dark jacket, is looking up at the word 'HOPE' written in large, white, block letters on a chalkboard. The chalkboard is dark and has some faint, light-colored markings. The word 'HOPE' is the central focus of the image. The woman is positioned in the lower right foreground, looking towards the word. The background is a dark chalkboard with some faint, light-colored markings. The word 'HOPE' is written in large, white, block letters. The woman is looking up at the word. The overall mood is hopeful and contemplative.

# HOPE

## Frankly Speaking About Cancer Clinical Trials

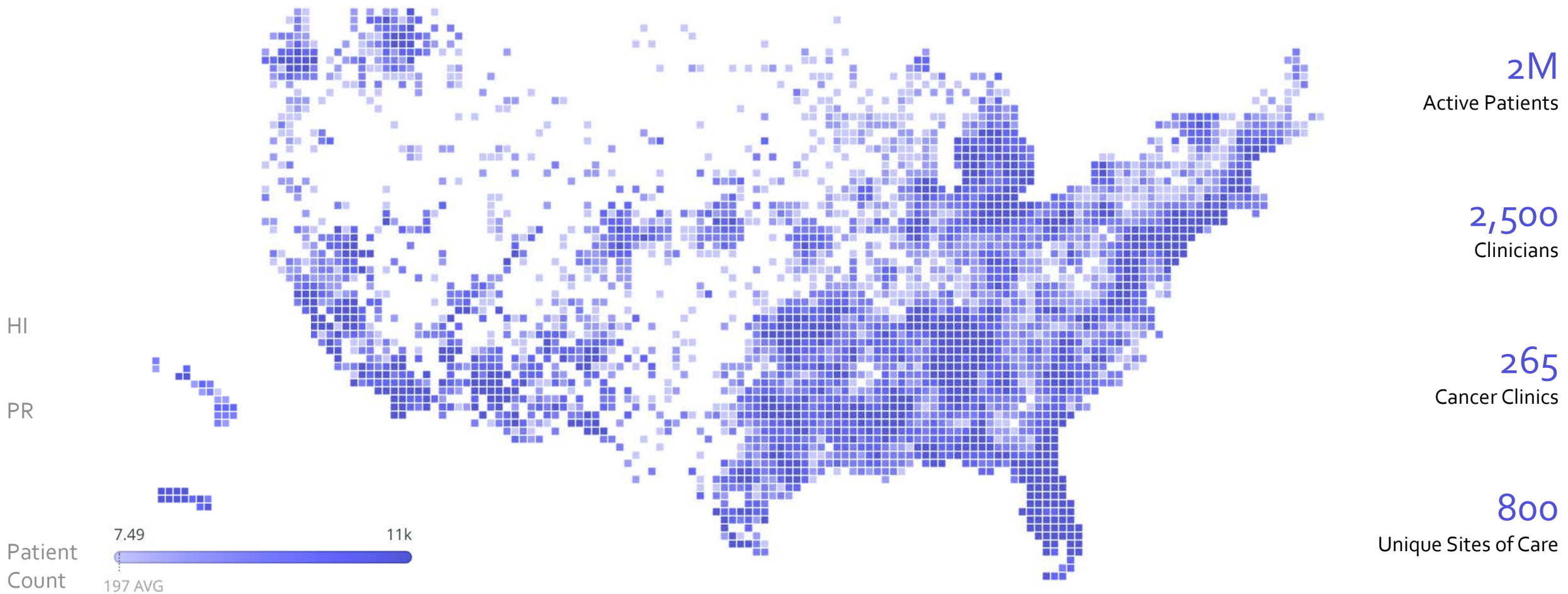
**FRANKLY<sup>®</sup>**  
SPEAKING  
| ABOUT CANCER

A PROGRAM *of the* CANCER  
SUPPORT COMMUNITY 

# Medical Review

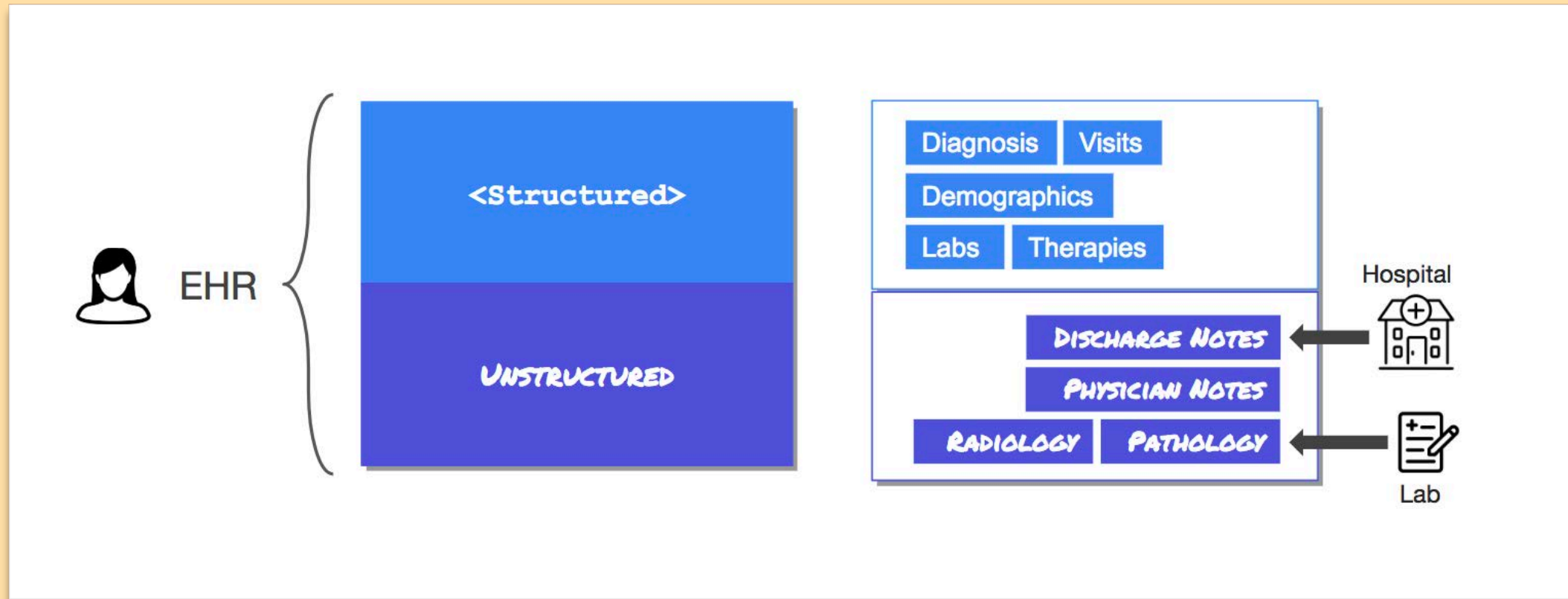
*The following slides were medically reviewed by  
Brad Hirsch, MD in February 2018.*

# Flatiron Health Network

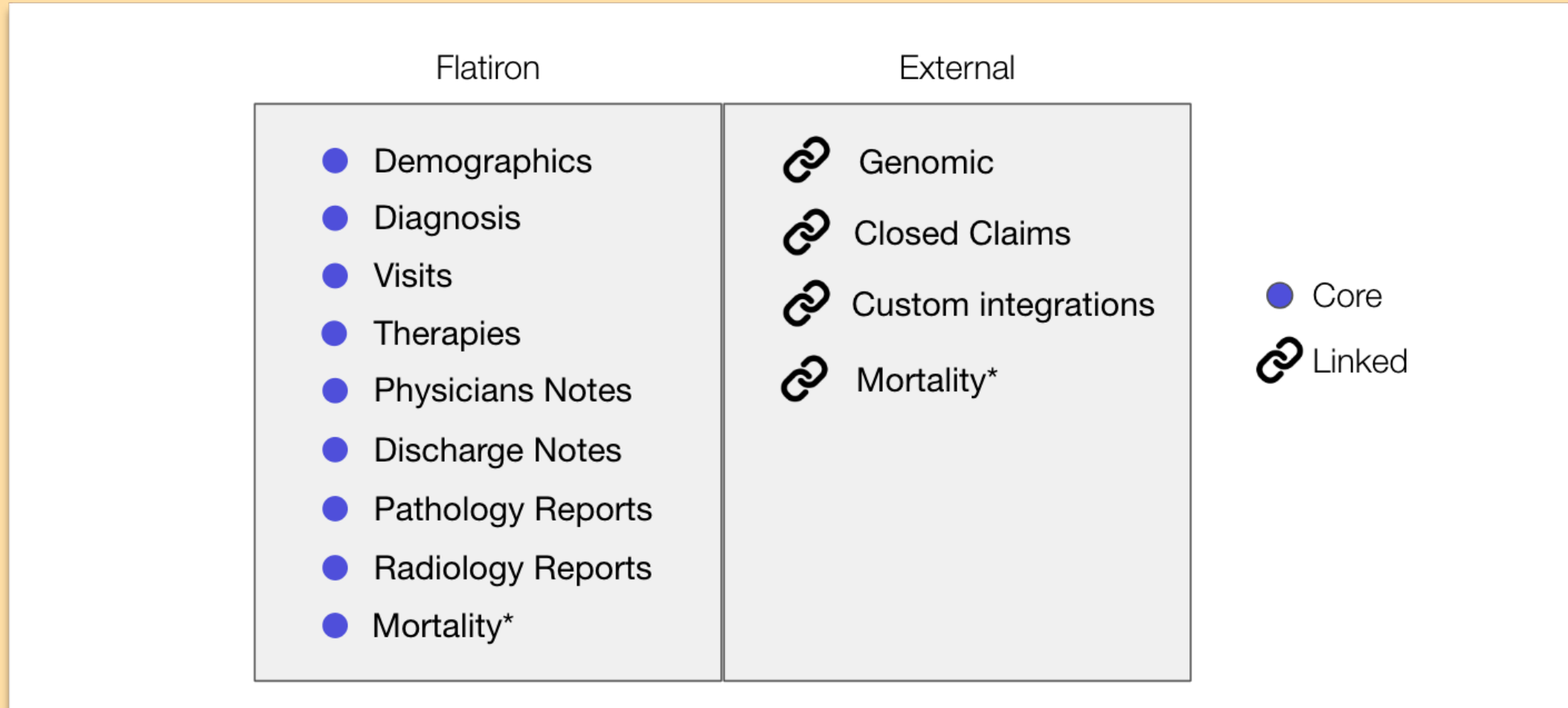




# Millions of electronic health records in a single common dataset



# Expanded with a linked dataset



# Workshop Overview

- What is a clinical trial
- Why clinical trials are important
- How trials work
- How to participate in a trial
- Why people don't participate
- The future—Rethinking the clinical trial model
- Communicating with your doctor about trials
- Questions

# Tips for Living in this Strange New Land

- You or your loved one may have been diagnosed with cancer, but that doesn't mean that “cancer has you”
  - Educate yourself
  - Get support
  - Have hope and courage



# Cancer Support Community Resources



Staffed by licensed professional counselors available to assist you Monday-Friday 9am-9pm ET. Also has a Clinical Trial Educator available by appointment.



When making treatment decisions, our Helpline specialists and local CSC and Gilda's Club staff can help you create a list of specific questions for your doctor about your concerns.



CSC's provides trusted information on a variety of topics important to people affected by cancer. Information is available through print and digital publications, online and in-person programs.



# What is a Clinical Trial?



A clinical trial is a research study that compares a new treatment or approach to the existing standard of care—the best treatment based on available evidence.

# Clinical Trials Are Done to:

- Help prevent cancers
- Diagnose cancers early
- Improve treatment for early stage tumors
- Reduce the risk of recurrence
- Improve treatment for advanced stage, rare, and difficult to treat tumors
- Reduce side effects from treatment
- Improve survival and quality of life

# Why Clinical Trials Are Important

- Due to advances from clinical research, two out of three people in the US live at least five years after a cancer diagnosis—up from roughly one out of two in the 1970s
- The nation's **cancer death rate has dropped 18 percent since the early 1990s**—reversing decades of increases
- Individuals with cancer are increasingly able to live active, fulfilling lives due to better management of symptoms and treatments with fewer side effects

# Why Clinical Trials Are Important

*“These dramatic trends, and the progress we are making, would have been unthinkable without the engine that drives life-saving cancer treatment—clinical cancer research.”*

*- Sandra Swain, MD, FACP, 2012 President of the American Society for Clinical Oncology (ASCO)*

# Why Clinical Trials are Important

- **Advanced Melanoma:** Over 40% of patients now achieving long-term remissions—or even cures with new therapies
- **Lung Cancer:** Patients with advanced disease living longer as a result of targeted and immunotherapies
- **Blood Cancers:** New T-cell therapies have 90% remission rates in people with advanced cancers
- **Pediatric Cancers:** The model for clinical trials. 80% of children with cancer cured of their disease
- **Breast Cancer:** Adjuvant therapies and treatment for metastatic disease have significantly increased survival rates



# Why Clinical Trials Are Important

- **Every** new treatment for cancer was tested in a clinical trial
- The **faster** that trials find participants, the faster that proven new treatments can get to patients
- **Today**, we are seeing rapid, dramatic progress in cancer treatment
- New therapies are based on **understanding** how cancers arise and grow
- **New models** allow for faster and more flexible evaluation of results
- There is more emphasis on **quality of life** and hearing what patients say about their experience

# But Few Adults Join Clinical Trials

- **Overall, only 4 percent of adult cancer patients ever participate in a clinical trial — a number that has not changed in years**
- Many people who could potentially benefit from trials are unaware of them
- Some groups of patients — minorities, older people, and even women — are still underrepresented in clinical trials
- Progress is slowed when trials are unable to recruit the patients they need to evaluate a new therapy



# Every Patient Should Know About Clinical Trials



Photo courtesy The James Comprehensive Cancer Center,  
The Ohio State University

*“I firmly believe that the best care for people with cancer is care received in a clinical trial, so I try to offer that option to every one of my patients.”*

*- David Carbone, MD, James Cancer Center at Ohio State University, Lung Cancer Expert*

# How Do Clinical Trials Work?

- Before being included in a trial, a new drug or agent
  - Undergoes extensive testing in the lab to understand how it works
  - Shows promise of activity
  - Does not demonstrate too much toxicity in animal models
- This is called preclinical research

# How Phase I Clinical Trials Work

- Phase I trials
  - First use in humans
  - Goal of the trial is to evaluate its safety, determine a safe dose and identify side effects
  - Safety means assessing the side effects or toxicity of the treatment
  - Usually in a small group of patients with different kinds of cancers



# What About Effectiveness?

- Phase I trials are not actually designed to test how well a new drug or combination of drugs works—but some patients who participate in the trial may benefit from the treatment.
- The results of the Phase I trial also provide important data to help decide the dose patients should receive in Phase II trials.

# How Phase II Clinical Trials Work

- Phase II Trials
  - Provide more information about the safety and efficacy of a treatment
  - Efficacy means does it work and is it better than the existing standard of care
  - Usually take about two years and involve larger numbers of patients
  - May involve randomization to different treatment approaches or arms

# How Phase III Clinical Trials Work

- Phase III Trials
  - Studies of treatments that have been promising in earlier trials in patients with a certain cancer type (or genetic mutation)
  - Large studies that often involve hundreds of patients in many cancer centers in the US and internationally
  - Usually randomized—to compare the new treatment to the existing standard of care
  - Have very specific measures for success or failure
  - Evaluate all side effects and problems that patients have during the trial
- Phase III trials provide the data the FDA uses to approve a new drug or treatment for a specific use.

# How Phase IV Clinical Trials Work

- Phase IV Trials
  - Evaluate side effects in the real world once a drug is approved for use in routine care



# Chimeric Antigen Receptor T Cells against CD19 for Multiple Myeloma

THE NEW ENGLAND JOURNAL of MEDICINE

## BRIEF REPORT

### Chimeric Antigen Receptor T Cells against CD19 for Multiple Myeloma

Alfred L. Garfall, M.D., Marcela V. Maus, M.D., Ph.D., Wei-Ting Hwang, Ph.D., Simon F. Lacey, Ph.D., Yolanda D. Mahnke, Ph.D., J. Joseph Melenhorst, Ph.D., Zhaohui Zheng, M.S., Dan T. Vogl, M.D., Adam D. Cohen, M.D., Brendan M. Weiss, M.D., Karen Dengel, R.N., B.S.N., Naseem D.S. Kerr, M.P.H., Adam Bagg, M.D., Bruce L. Levine, Ph.D., Carl H. June, M.D., and Edward A. Stadtmauer, M.D.

## SUMMARY

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Drs. Garfall and Maus contributed equally to this article.

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A patient with refractory multiple myeloma received an infusion of CTL019 cells, a cellular therapy consisting of autologous T cells transduced with an anti-CD19 chimeric antigen receptor, after myeloablative chemotherapy (melphalan, 140 mg per square meter of body-surface area) and autologous stem-cell transplantation. Four years earlier, autologous transplantation with a higher melphalan dose (200 mg per square meter) had induced only a partial, transient response. Autologous transplantation followed by treatment with CTL019 cells led to a complete response with no evidence of progression and no measurable serum or urine monoclonal protein at the most recent evaluation, 12 months after treatment. This response was achieved despite the absence of CD19 expression in 99.95% of the patient's neoplastic plasma cells. (Funded by Novartis and others; ClinicalTrials.gov number, NCT02135406.)

**T**RANSDUCTION OF AUTOLOGOUS T CELLS TO EXPRESS CD19-SPECIFIC CHIMERIC antigen receptors is a promising immunotherapeutic approach for the treatment of B-cell cancers.<sup>1</sup> We previously reported sustained regression of refractory chronic lymphocytic leukemia and B-cell acute lymphoblastic leukemia<sup>2-5</sup> after infusion of CTL019 cells, which consist of autologous T cells expressing a CD3-zeta/CD137-based anti-CD19 chimeric antigen receptor from a lentiviral vector. Multiple myeloma is a B-lineage cancer that is reported to express CD19 in



# How Clinical Trials Work

- Most later phase trials are designed to evaluate:
  - Overall survival (OS)—do patients on the new treatment live longer than those on the standard of care
  - Progression free survival (PFS)—how long does it take from the time treatment starts until the cancer begins to grow again
  - Disease free survival (DFS)—how long does it take before there is evidence that the cancer has recurred or metastasized
  - Complete remission (CR) – Does the treatment result in complete disappearance of the cancer?

# Participating in a Clinical Trial

- Every person facing cancer should talk to his/her doctor about whether a clinical trial is an option

# Informed Consent & Asking Questions

- Everyone who participates in a trial provides “informed consent” before they begin the study. This means your team will go over every aspect of the trial with you—and answer all your questions.
- You can take time to think about the trial and talk to your family.
- You can request another session to ask more questions
- You can stop participating in the trial at any time
- You can ask to see the results of your trial, or of other similar trials

# Participating in a Clinical Trial

*“We knew from the beginning that a clinical trial was the way to go. My cancer is rare and I was diagnosed with stage IV disease. Standard therapy didn’t offer much hope. We started looking right away and were willing to go anywhere where they had trials for my cancer.”*

*- Shannon, Stage IV cancer of the thymus, now in remission after immunotherapy clinical trial*

# How Do You Find a Trial?



- Talk to your doctor and treatment team
- Get your treatment in a cancer center that participates in clinical trials
- Do your own search



# Searching for Trials

- Online
  - Use established resources, like [clinicaltrials.gov](https://clinicaltrials.gov)
  - Go to websites for advocacy groups that offer clinical trial search help
  - Go to cancer center websites
- Communication with your doctor is key

# Finding a Clinical Trial

- Know your specific cancer type, stage and any genetic profiling information
- Just be aware that it can be very hard to find a trial that is right for you due to:
  - Complicated language
  - Very specific eligibility requirements
- However, by using available resources and your doctor, opportunities can often be found

# Finding a Clinical Trial



*“My wife was a market researcher and very good at finding information on line, but we were completely confused when we searched for clinical trials for her triple negative breast cancer. We took what we found to our doctor and really relied on her to separate real possibilities from what we had on paper.”*

*- Mark, husband and caregiver*

CANCER SUPPORT COMMUNITY

# So, Why Don't People Participate?

## The “guinea pig” myth

- Clinical trials are experimental studies. They are done to find out new information about cancer treatment—but people who participate are not guinea pigs. They receive the highest level of care available.
- All trials in the United States require that the clinical team truly believes that the therapy being tested might be better than the standard of care in order to move forward with the trial

# Why People Don't Participate

## The placebo issue

- Everyone in a clinical trial receives either the standard of care or the new approach. There are very few situations in cancer care today in which the standard of care is a placebo. You can always ask your doctor about the treatment arms.



# Why People Don't Participate



## Changing doctors or treatment centers

- Some trials do require that you change doctors or treatment centers. Your doctor should help make that connection. This can be worthwhile if another center offers a study that is a potentially good option for your cancer.

# Why People Don't Participate

*“I spend a lot of time talking to my colleagues in other centers to find trials for my patients—and they send us patients when we have a study that is a good option for their patient. No one place can have trials for everyone. Sometimes, my patients think I am abandoning them when I suggest they leave me to be part of a trial. I always reassure them that I am doing what is best for them—and that they can always come back.”*

*-Crystal Denlinger, MD, Fox Chase Cancer Center, gastric cancer specialist*

# Why People Don't Participate

## Cost

- The costs of care for people in a clinical trial is covered, either by insurance or the trial's sponsor. If you encounter other costs, talk to our doctor.



# Why People Don't Participate

Suspicion or distrust of the medical profession

- Historical and cultural events have led some groups, especially minorities to be suspicious of the medical profession. Today, it is much more important to make sure that everyone has access to clinical trials and the best treatment.

# Why People Don't Participate

*“I treat a lot of minority patients with cancer on trials. I know that I when I talk to a patient, I am talking to the family, and the community and I have to be aware of the factors that caused people not to trust doctors or clinical trials. I need to take the time to sit down and work through these issues.”*

*- Craig Cole, MD, University of Michigan, multiple myeloma specialist*



# Why People Don't Participate

No one ever talks to them about clinical trials

- Have the conversation with your doctor
- Ask questions
- Be willing to get a second opinion



# Why People Don't Participate

## “The last ditch treatment”

- While it is true that many clinical trials are done for late stage cancers, or for cancers that have stopped responding to other treatments, there are trials for every stage of cancer. Some are designed to improve outcomes for earlier, high risk cancers. Others are focused on reducing side effects or improving quality of life.

# Why You Should Participate in a Trial

- Hope
- Getting access to the newest, most innovative therapies
- Excellent care and monitoring
- A chance to have your voice heard
- Contributing to the greater good and a better future

# Why You Should Participate in a Trial



*“I chose the trial because I really hoped it would help my cancer, but I also felt very strongly that I was doing something important. If it didn’t work for me, it would help someone else. It would help people in the future. That meant a lot to me.”*

*- Ide, lung cancer patient*

# Communicating with Your Doctor

- Be open to the option of a clinical trial
- If your doctor doesn't bring it up, ask
- Make a list of your questions
- Talk to the nurse practitioners, nurses and coordinators—they are great resources
- Take time to think about the trial
- Don't hesitate to get a second opinion
- If you look for trials on your own, discuss what you find with your doctor



# The Future

- New Models of Clinical Trials
  - Targets not organs—Trials focused on specific genetic mutations rather than cancer types
  - More flexibility in trial design
  - More effective therapies—better patient participation
  - More input from patients and advocates in designing and evaluating trials



# CANCER EXPERIENCE REGISTRY

- Provides a unique online community
- Identifies specific issues—emotional, physical and social
- Gives voice to the individual and collective experience
- Shares experience—both to learn from and help others
- Encourages self-advocacy
- Encourages active participation in the cancer community
- Provides critical data to educate the medical community
- Provides evidence for policy makers
- Guides program/service development
- Helps improve quality of care



# CANCER EXPERIENCE REGISTRY

## How to Join?

- Go to [www.CancerExperienceRegistry.org](http://www.CancerExperienceRegistry.org)
- Submit your contact information on the participant evaluation at the end of this workshop
- Explore the website
- Register by developing a secure login
- Complete a questionnaire to share your experience
- Receive expert education materials and resources



# CANCER EXPERIENCE REGISTRY

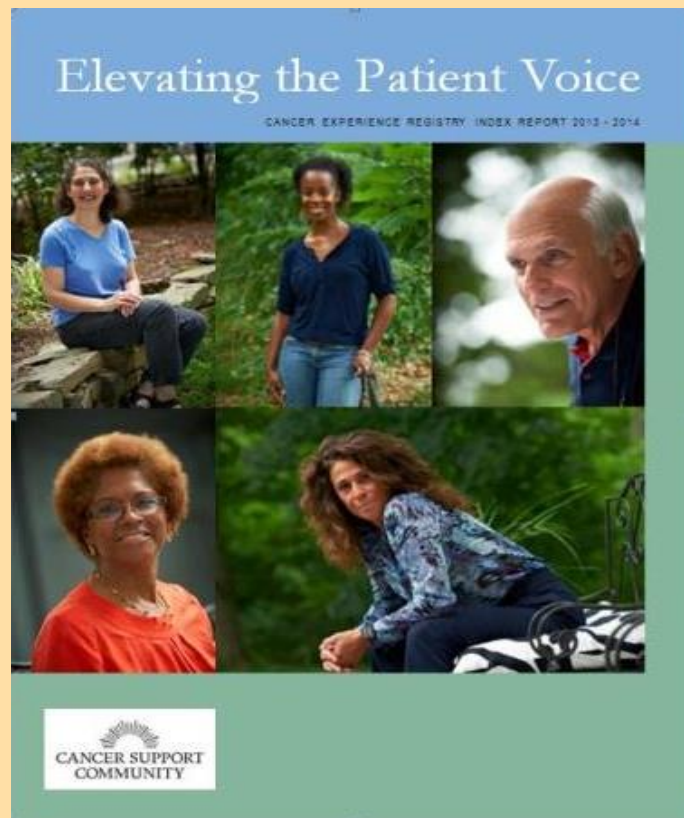
- Open to anyone impacted by cancer and their caregivers
- Confidential and secure
- Identifies issues specific to the cancer experience
- Shares experience—both to learn from and help others
- Includes sub-registries for specific cancer diagnoses:
  - Breast Cancer
  - Metastatic breast cancer
  - Multiple myeloma
  - Chronic myeloid leukemia
  - Chronic lymphocytic leukemia
  - Lung cancer
  - Melanoma
  - Prostate cancer
  - Caregivers
  - Stomach cancer
  - Others being added





# CANCER EXPERIENCE REGISTRY

## Annual Index Report (2014)



## What We Have Learned

### TOP CONCERNS OF CANCER EXPERIENCE REGISTRY PARTICIPANTS

- *Eating and nutrition*
- *Worrying about the future and what lies ahead*
- *Health insurance or money worries*
- *Feeling too tired to do the things you need or want to do*
- *Exercising and being physically active*
- *Worrying about family, children and/or friends*
- *Changes or disruptions in work, school or home life*
- *Thinking clearly (e.g., "chemo brain")*
- *Moving around  
(walking, climbing stairs, lifting, etc.)*
- *Body image and feelings about how you look*
- *Sleep problems*

# Join Our Grassroots Movement

- Help ensure that people touched by cancer have access to quality, comprehensive cancer care that includes social and emotional support.
- This community will provide you the opportunity to:
  - Get up-to-date information on key issues that are important to patients with cancer and their loved ones
  - Be part of a network that interacts with Capitol Hill and other policymakers on important issues to cancer patients
  - Have your voice heard along side other voices of patients with cancer and their loved ones
  - **To join, visit:** [www.CSCAdvocate.org](http://www.CSCAdvocate.org)





# Questions & Answers



This program was made possible with support from:



**Genentech**