

# How LLS is changing the landscape of blood cancer

Rob Dean, MD

Cleveland Clinic Taussig Cancer Institute

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LYMPHOMA  
SOCIETY®

fighting blood cancers

# Our Mission:

## 3 focus areas:

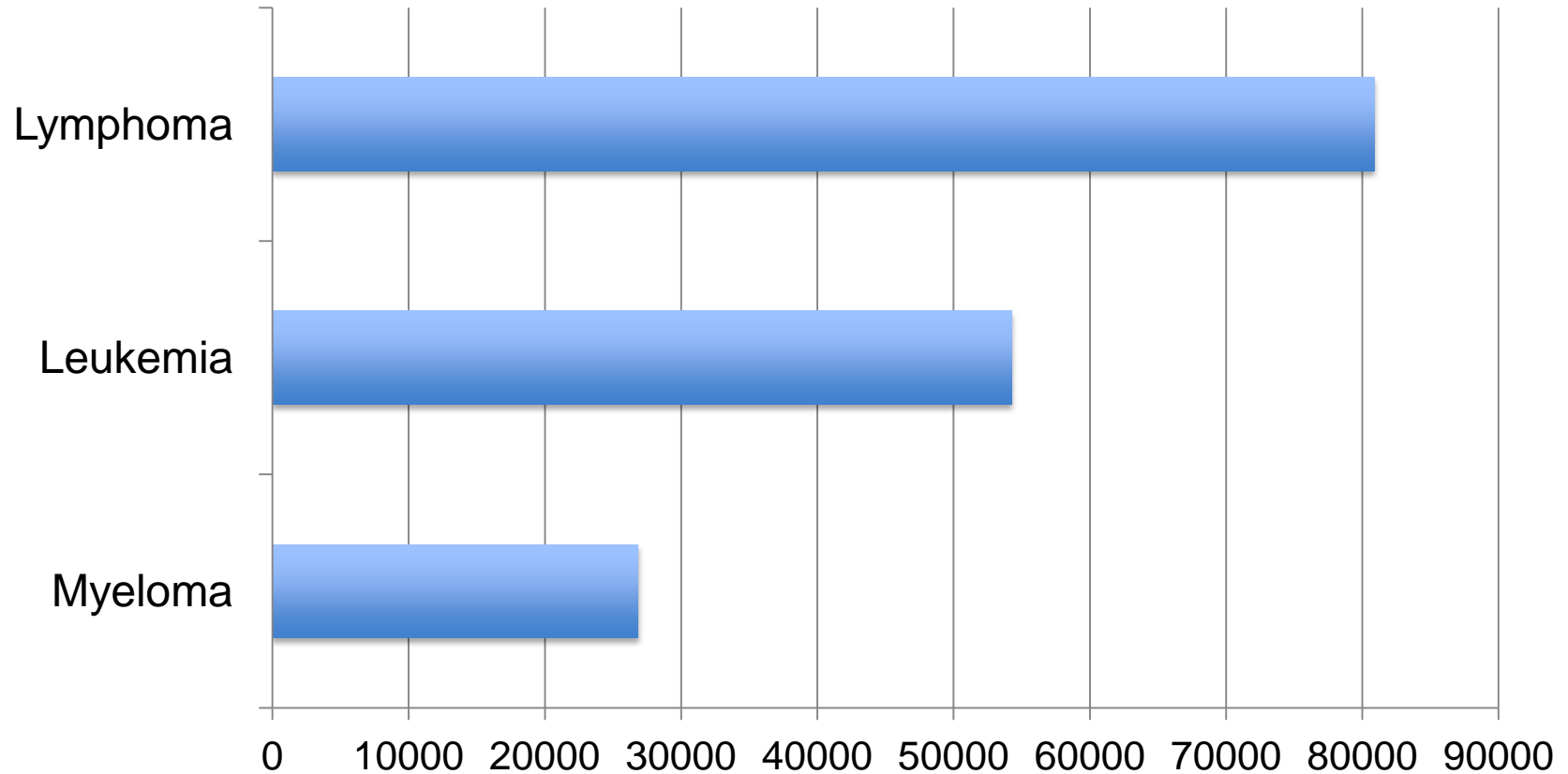
- Research
- Patient access
- Advocacy

Cure leukemia,  
lymphoma,  
Hodgkin's  
disease,  
and myeloma,  
and improve  
the quality of life  
for patients  
and their families.

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# Blood cancers are **almost 10%** of new cancer diagnoses

162,000 new cases in 2015 (U.S.)



# Blood cancers are the number three cancer killer

Every three minutes someone is diagnosed with a blood cancer. Every ten minutes someone dies from a blood cancer.

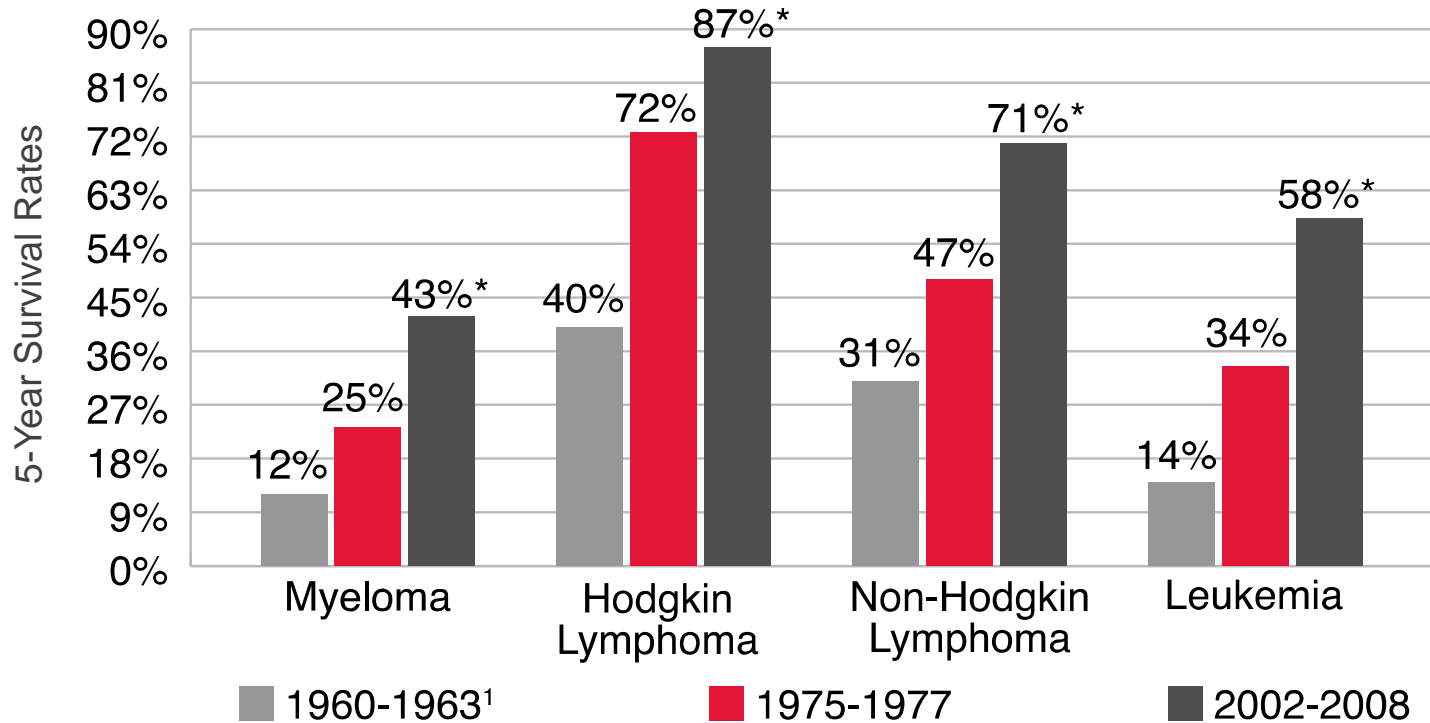
But, we are making tremendous progress.



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Since the 1960s, the survival rates for many blood cancer patients have **doubled, tripled** and even **quadrupled**



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# More work needs to be done

Despite progress, more than a third of blood cancer patients still do not survive five years after their diagnosis.



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# LLS exists to **find cures** and **ensure access to treatments** for blood cancer patients

There are no means for preventing or early screening for most blood cancers.

Therefore, LLS focuses on finding cures and ensuring sustainable access to quality, affordable, coordinated care.



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# Once unimaginable, new treatments are **saving lives today**

Moving from highly toxic treatments to more targeted therapies...

**1950s**

First chemotherapy agents for lymphoma and leukemia patients, including children

**1960s**

First combination chemotherapy developed for childhood leukemia

**1970s**

First successful bone marrow transplants performed

**1980s**

Cancer-causing oncogenes and tumor suppressor genes discovered

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# Once unimaginable, new treatments are **saving lives today**

... with cures and prevention as the ultimate long-term goal

**1990s**

Antibody-based therapies such as Rituxan

**2000s**

Targeted therapies such as Gleevec

**2010s**

Genomic medicine and precision medicine; adoptive immunotherapy

**2020 and beyond**

Personalized medicine  
Cures and prevention

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# New drug approvals for blood cancers, 2010-present

| Year | Disease  | Drug   |
|------|--|--|
| 2010 | CML  | Dasatinib, Nilotinib   |
| 2011 | Myelofibrosis<br>ALL<br>Hodgkin lymphoma<br>Anaplastic large cell lymphoma           | Ruxolitinib<br>Erwinia asparaginase<br>Brentuximab vedotin<br>Brentuximab vedotin            |
| 2012 | CML<br>ALL<br>Multiple myeloma   | Ponatinib, Omacetaxine mepesuccinate, Bosutinib<br>Vincristine liposome<br>Carfilzomib       |
| 2013 | Mantle cell lymphoma<br>CLL<br>Multiple myeloma                                      | Ibrutinib, Lenalidomide<br>Obinutuzumab<br>Pomalidomide                                      |
| 2014 | CLL<br>Follicular lymphoma<br>Peripheral T-cell lymphoma<br>Polycythemia vera<br>ALL | Ibrutinib, Ofatumumab, Idelalisib<br>Idelalisib<br>Belinostat<br>Ruxolitinib<br>Blinatumomab |
| 2015 | Multiple myeloma<br>Lymphoplasmacytic lymphoma                                       | Panobinostat<br>Ibrutinib  |

Source: [www.fda.gov](http://www.fda.gov)

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# Access Policy: Challenge and Opportunity

How many lives will be saved by rituximab, imatinimb or even newer drugs if patients:

- Can't afford the out-of-pocket costs to fill their prescription?
- Have insurance that doesn't cover these treatments?
- Don't have adequate insurance coverage?
- Can't navigate the healthcare system to get access to the care they need?

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# Committed to Improving Patients' Quality of Life

LLS provides free information and support services for patients and their families.

Our Co-Pay Assistance program has provided more than \$197 million since inception.



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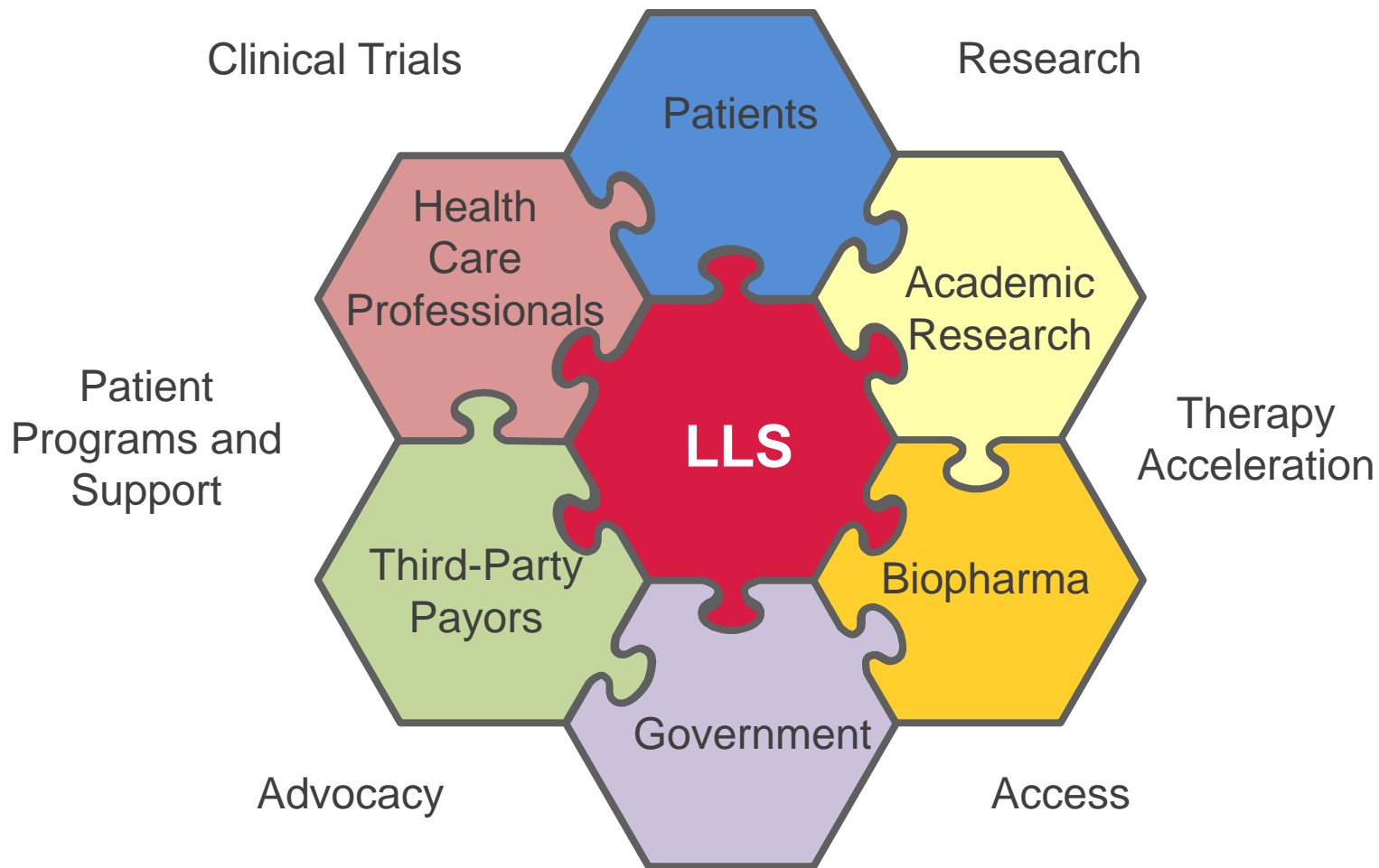
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# Research at LLS today

research

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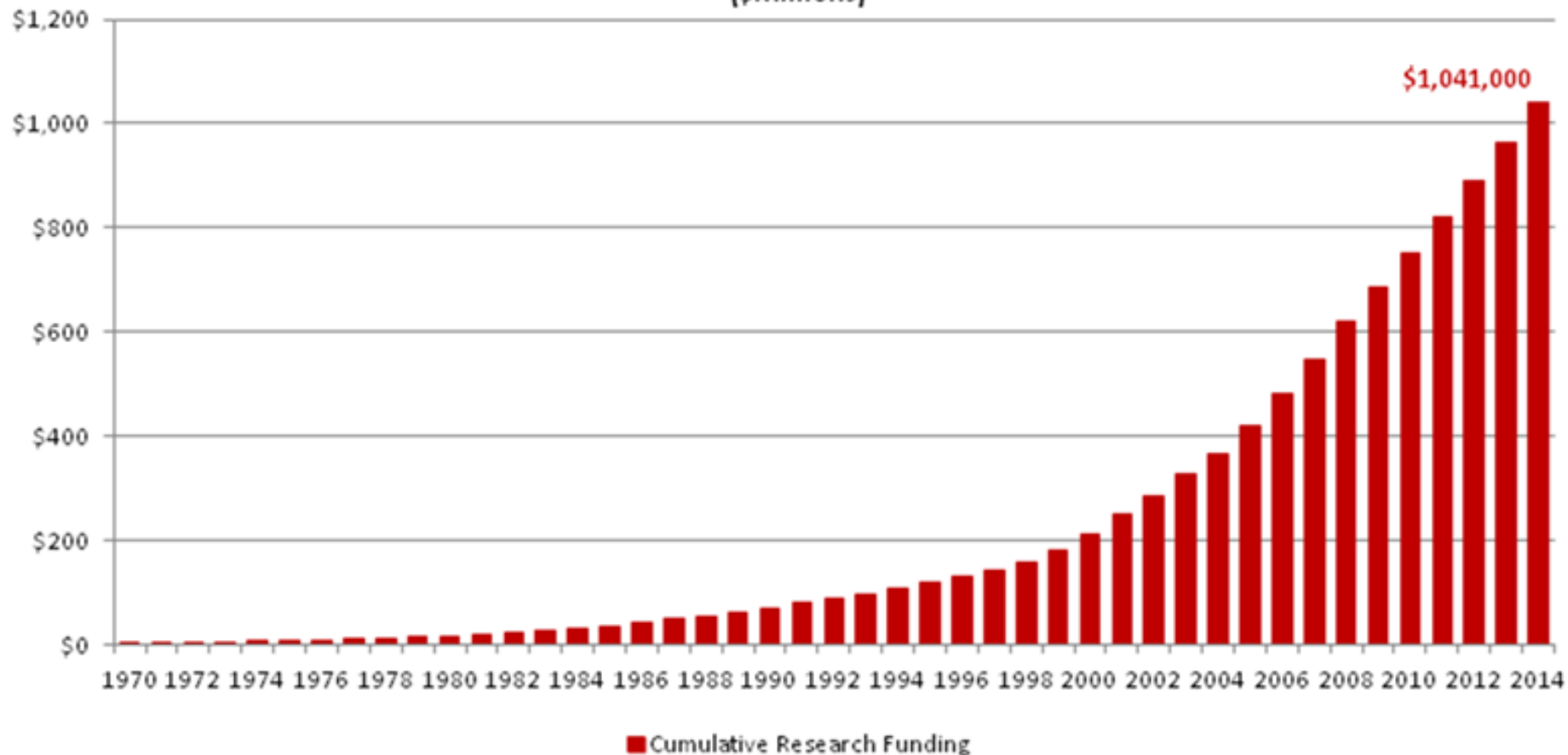
# Aligning the Players in the Innovation Ecosystem



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# LLS: Over \$1 Billion in Research Funding

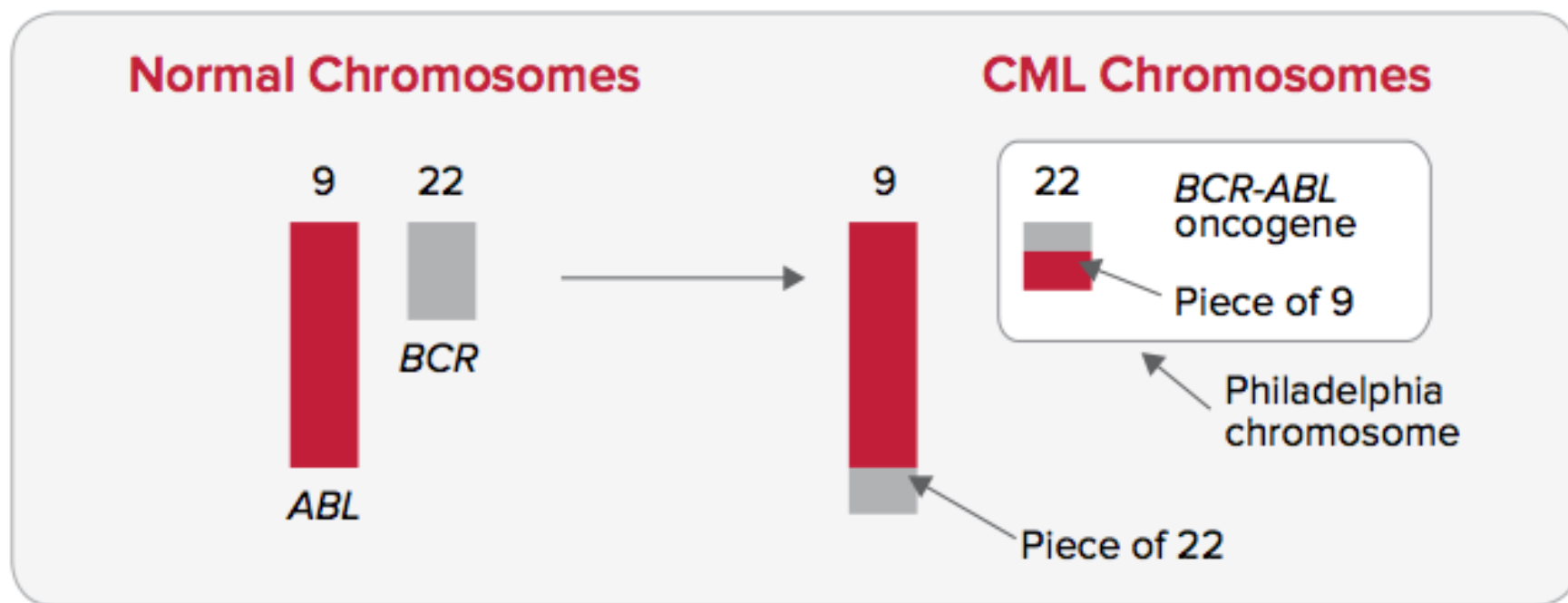
## Cumulative Research Funding (\$Millions)



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# CML has a consistent molecular target

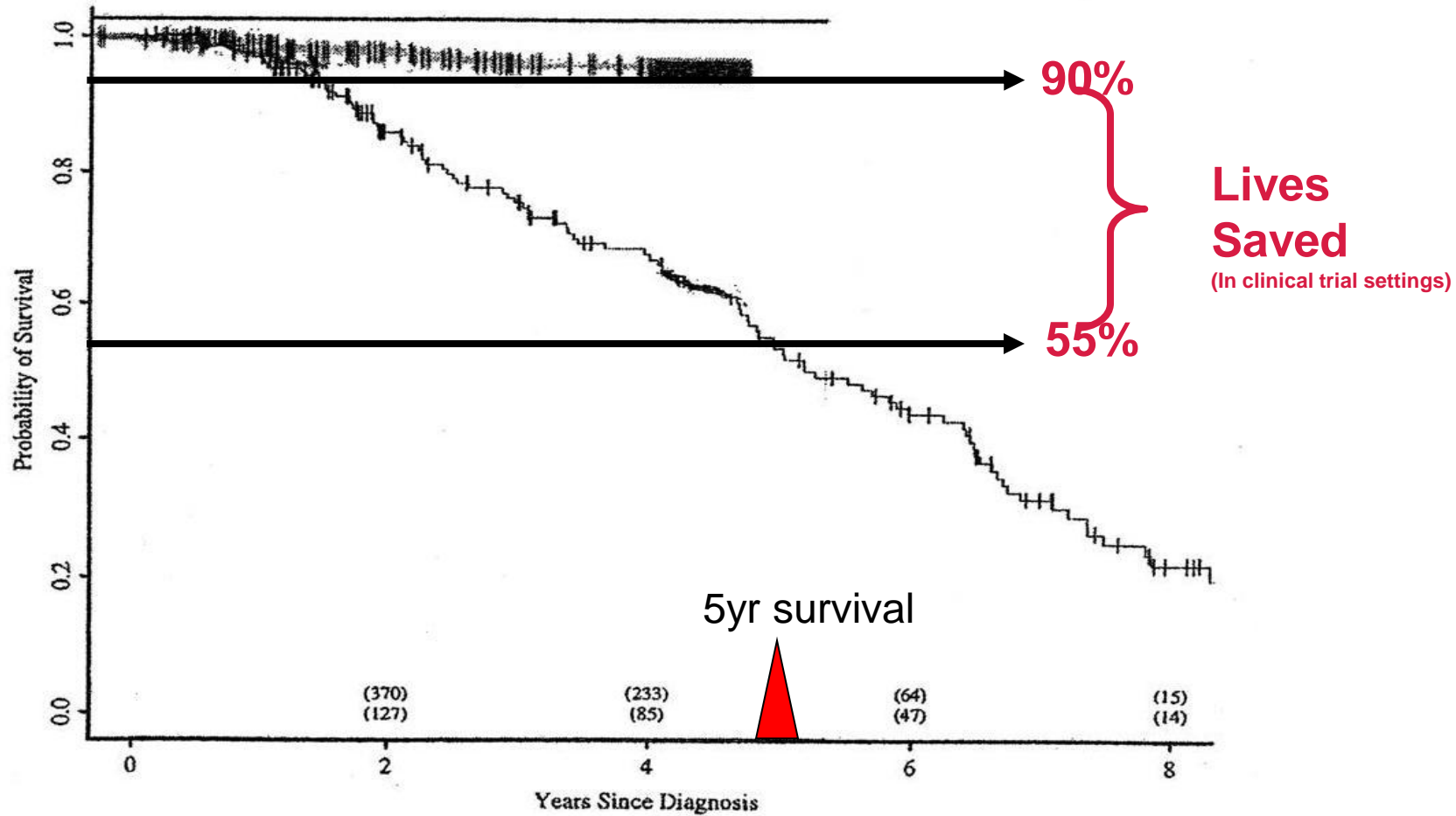
## Translocation of chromosomes 9 and 22



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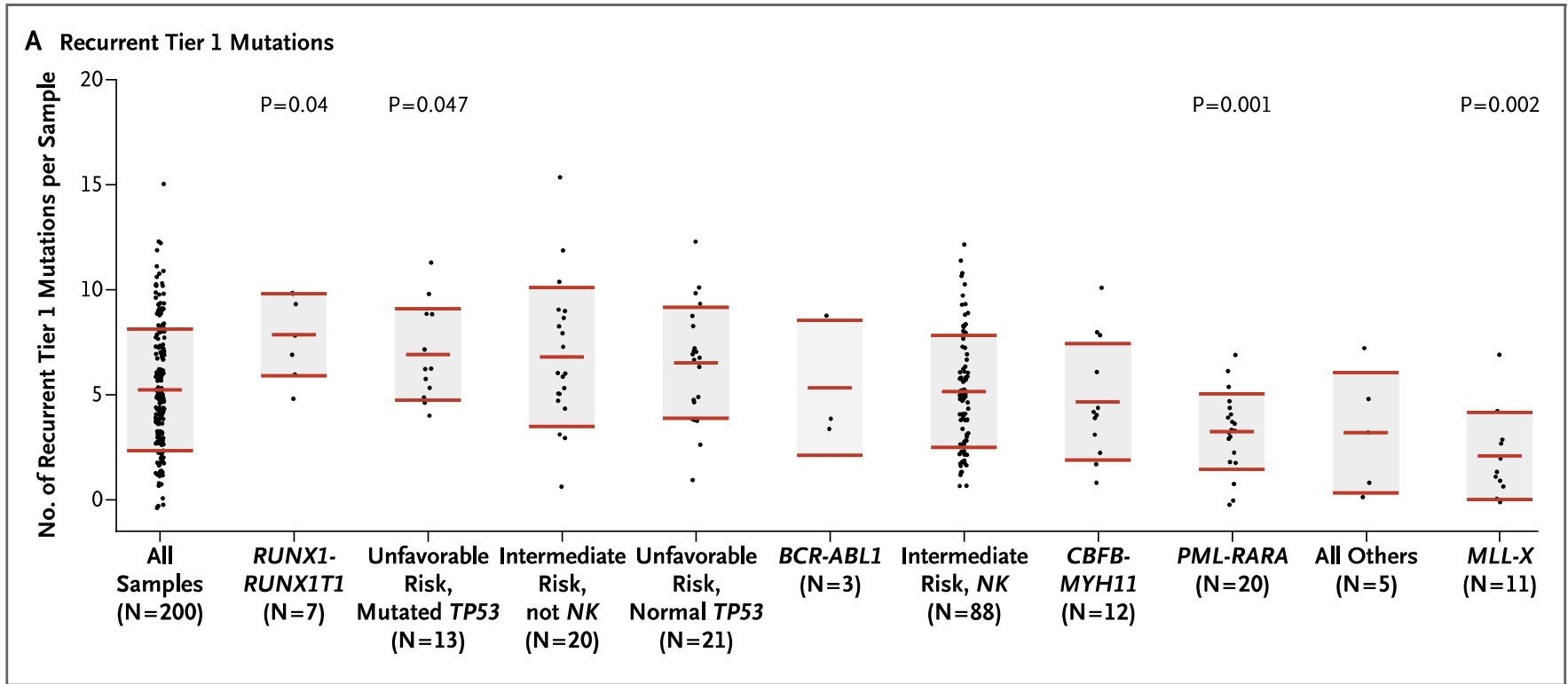
# CML: lives saved due to imatinib



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# Most blood cancers are genetically complex

## Average of 5 recurring mutations per case in AML



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# Leading in Venture Philanthropy

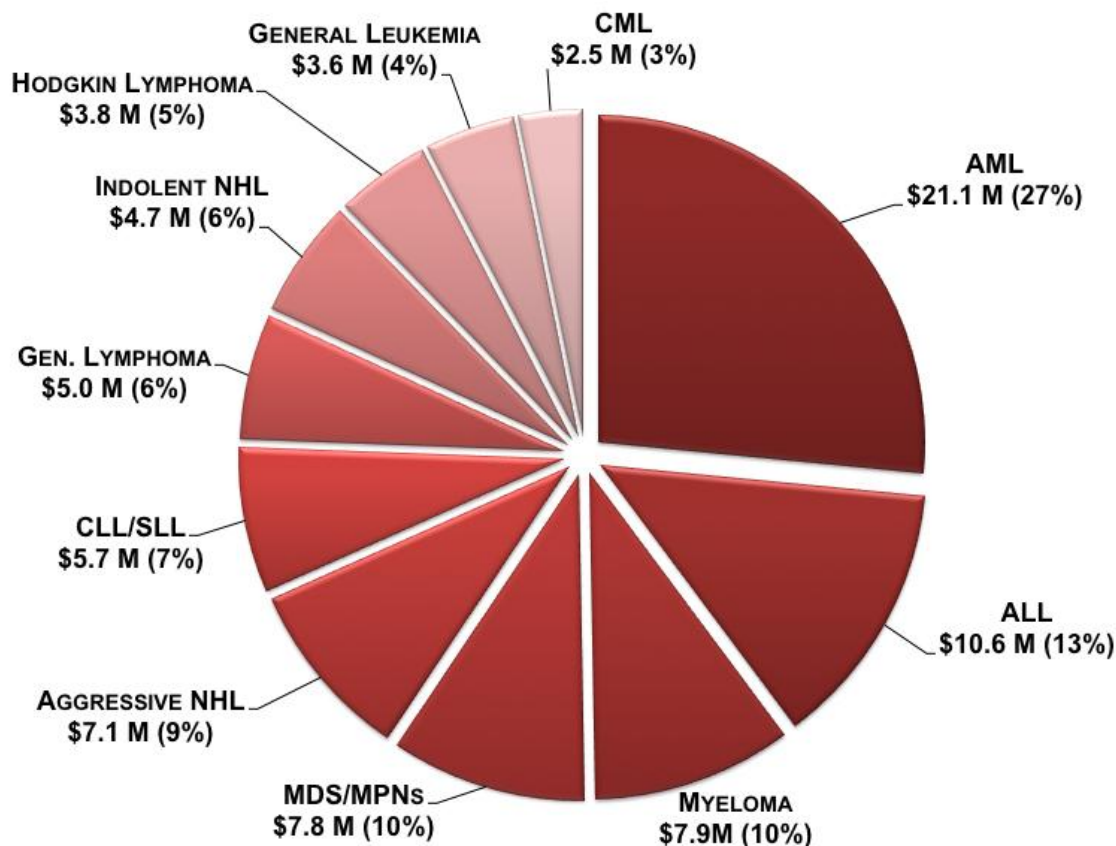
LLS partners with universities, hospitals, and biotechnology and pharmaceutical companies to get treatments to patients faster than ever.



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# FY14 Research Commitment

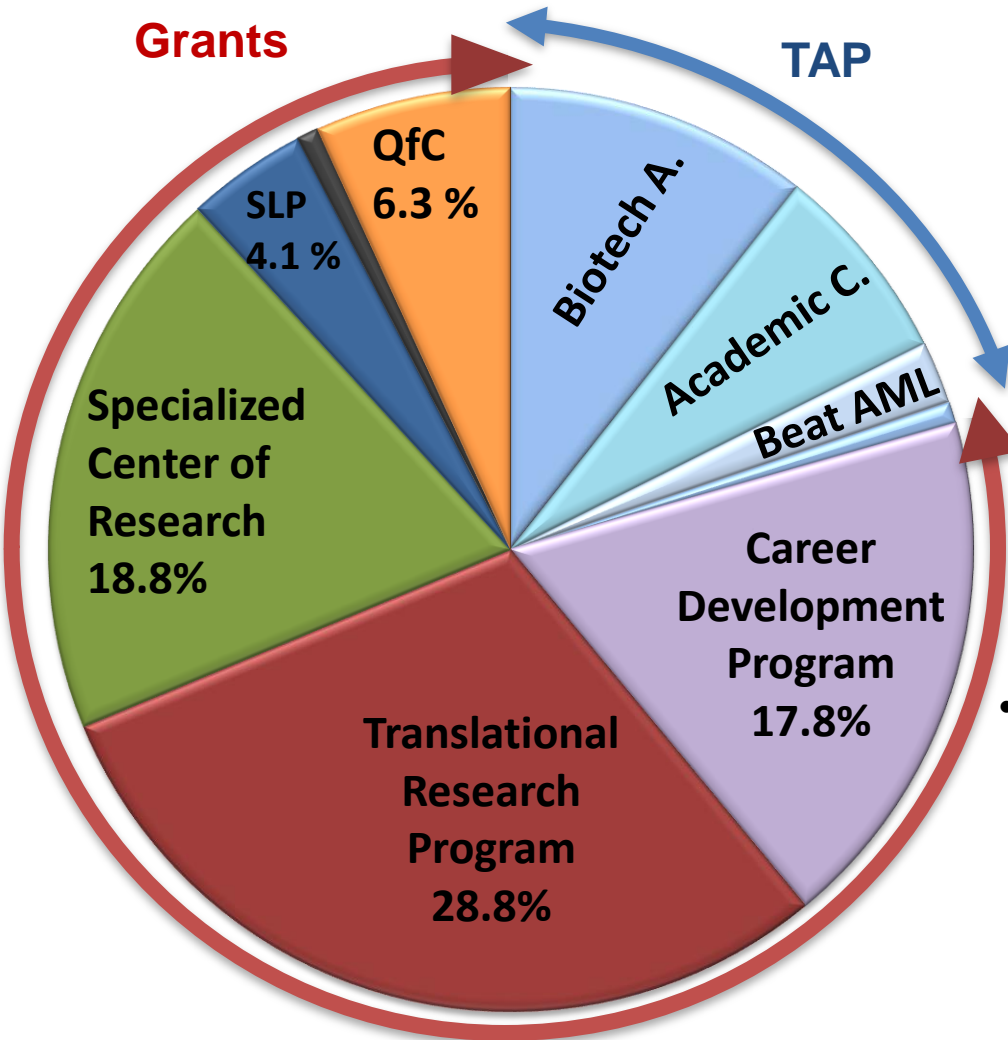


**Research Budget: \$79.8 Million**



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# LLS Current Research Portfolio



## 333 Active Academic Grants

- Career Development (CDP) – “training award”
- Translational Research (TRP) – “bench to bedside”
- Specialized Center of Research (SCOR) – synergistic collaboration
- New Idea Award (NIA) – “crazy idea, concept”
- Screen to Lead (SLP) – “finding leads”
- Quest for CURES (QFC) – focused
- Other partnerships – IWMF & MPNRF

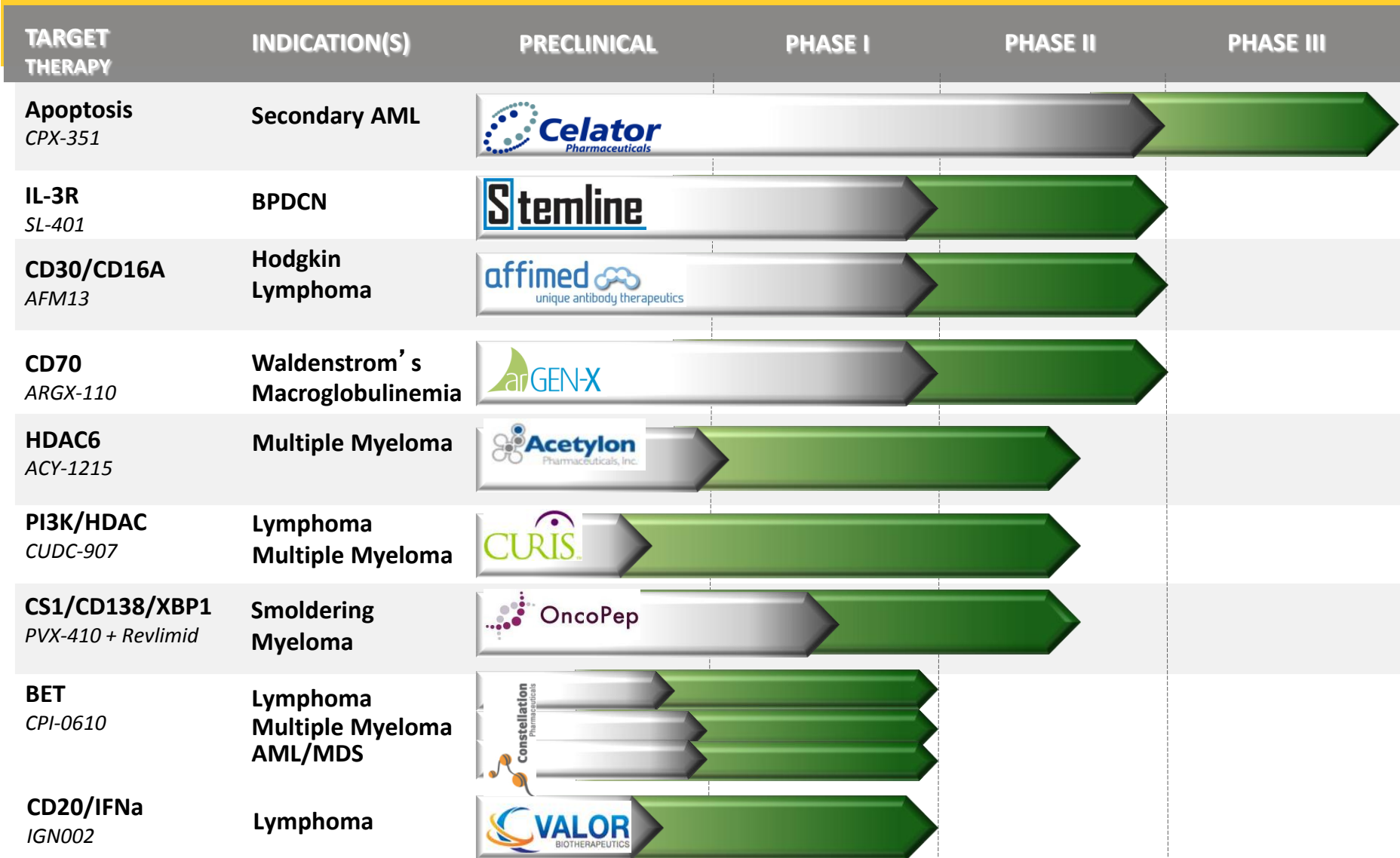
## • 25 Therapy Acceleration Programs

- Goal is to accelerate first in class opportunities
- Pre-IND to Phase 3 studies
- Concentrated in “valley of death”























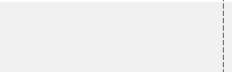

FY14: \$79.8 M

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# Biotech Accelerator TAP Pipeline



# Academic Concierge TAP Pipeline

| TARGET THERAPY                                | INDICATION(S)              | PRECLINICAL  | PHASE I   | PHASE II   | PHASE III |
|---|----------------------------|--|---|--|-----------|
| <b>Cell Therapy</b><br><i>MiLs</i>            | <b>Multiple Myeloma</b>    |   |    |   |           |
| <b>Hedgehog</b><br><i>PF-04449913</i>         | <b>Acute Leukemias/MDS</b> |   |    |   |           |
| <b>local RT + CTLA-4</b><br><i>Ipilimumab</i> | <b>Lymphoma</b>            |   |    |   |           |
| <b>VDA</b><br><i>Oxi4503</i>                  | <b>AML/MDS</b>             |   |    |   |           |
| <b>CDA/DNMT1</b><br><i>THU-Decitabine</i>     | <b>AML/MDS</b>             |   |    |   |           |
| <b>CDC7</b><br><i>MSK-777</i>                 | <b>Acute Leukemias</b>     |    |   |   |           |
| <b>Menin</b><br><i>Sm Molecule</i>            | <b>MLL Leukemias</b>       |   |  |   |           |

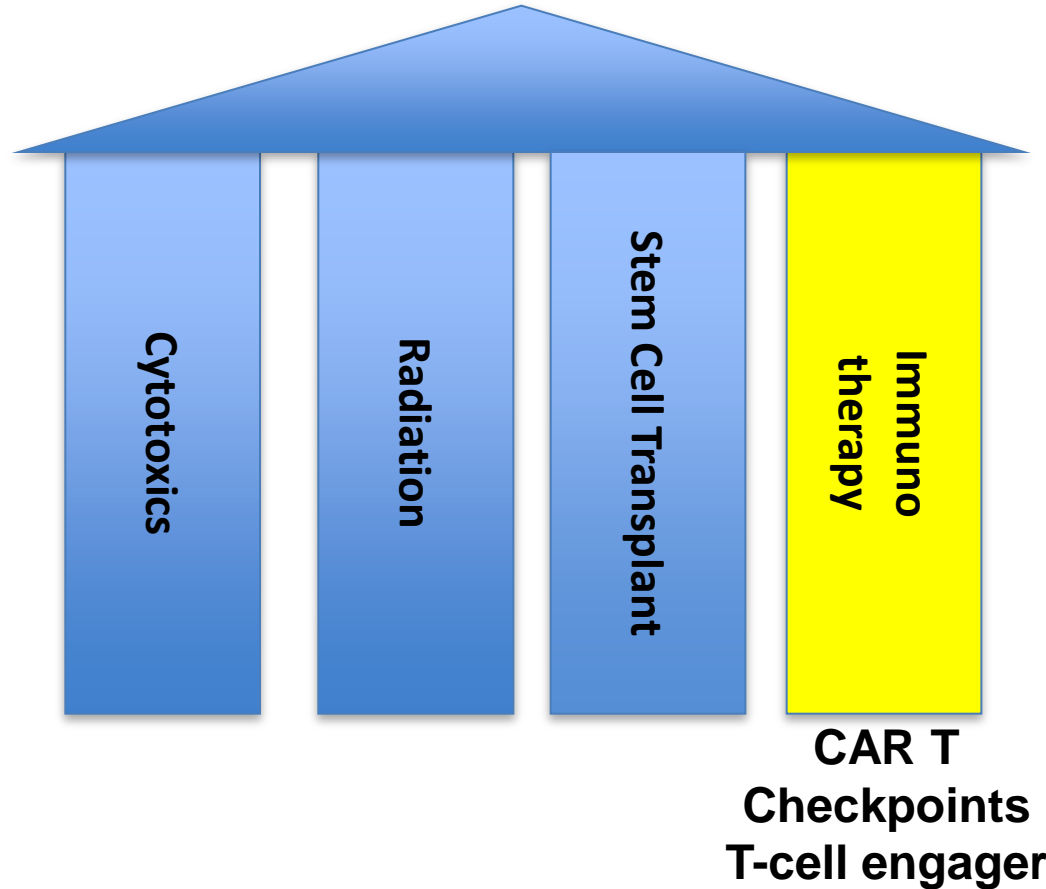
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# Two Transformational Years for Immunotherapy to Treat Blood Cancer



## Building a New Foundation for Blood Cancer Therapy



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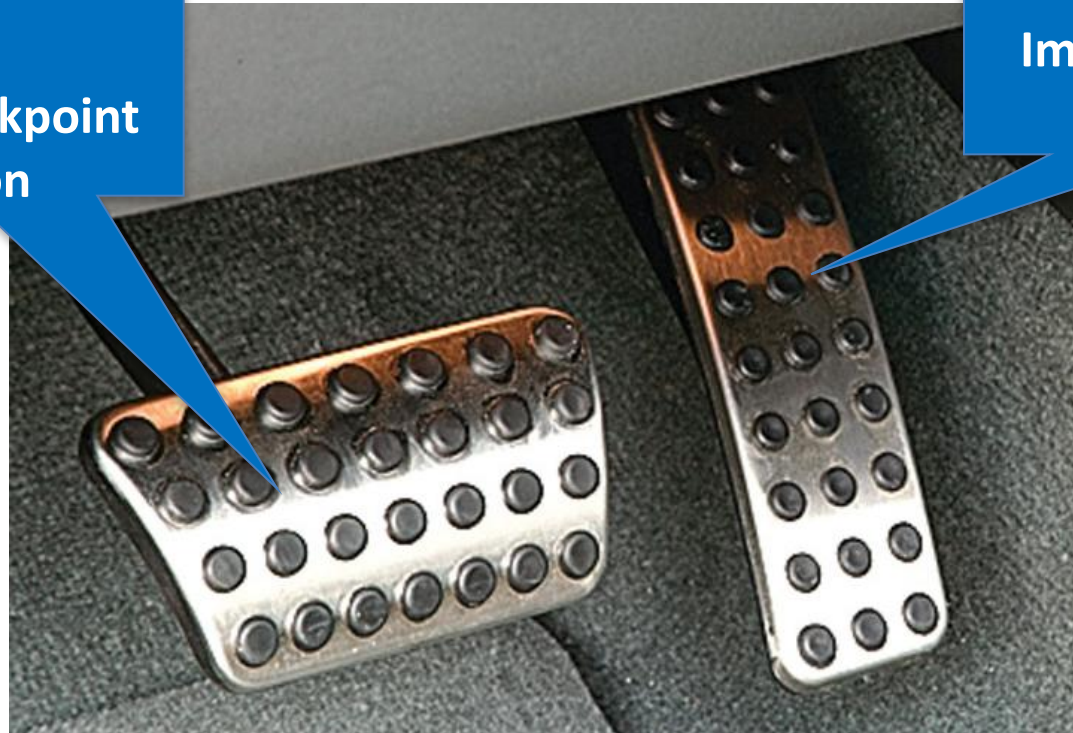
# Activation of The Immune System by Two New Methods

**Release the  
brake:**

**Immunecheckpoint  
inhibition**

**Step on the gas**

**Immunoactivation  
(CAR T)**

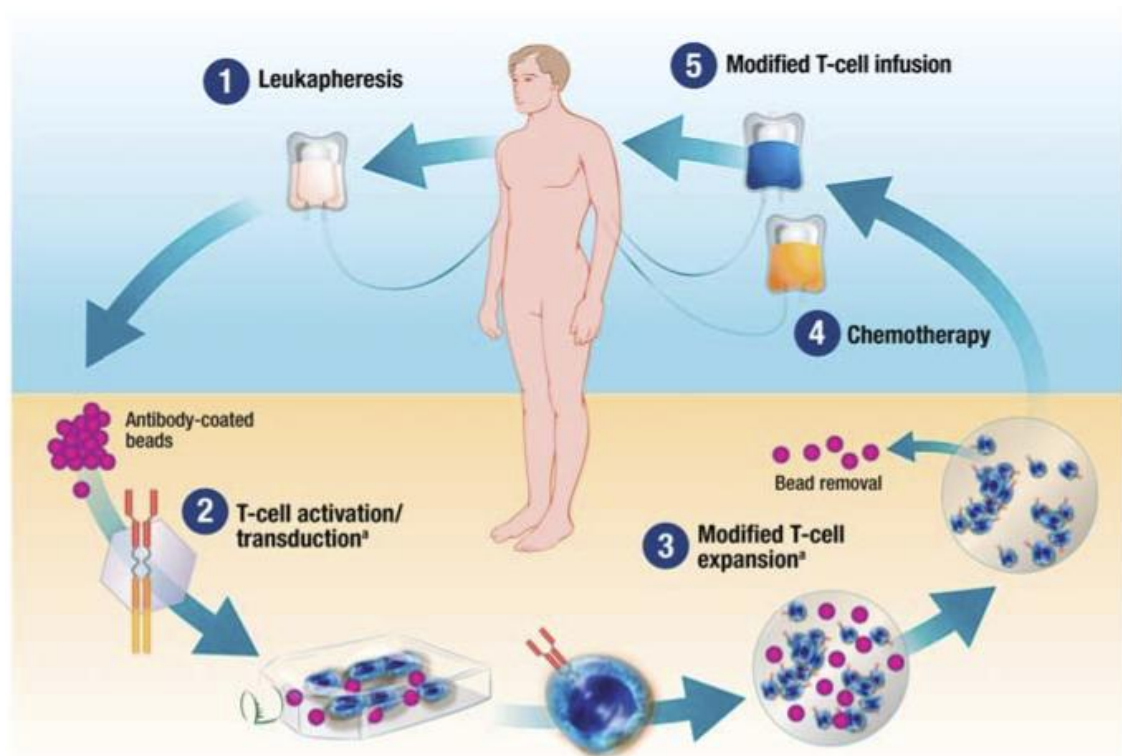


1. From Chen et al., 2013 (LLS investigator)

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# CAR T-cell immunotherapy

- *Chimeric antigen receptor (CAR)* engineered T-cells
  - Redirects immune cells to attack cancer cells
- ALL, CLL, NHL
- Potential use in many cancers



# Immune checkpoint inhibitors for Hodgkin lymphoma

**Disease: Hodgkin lymphoma**

**Therapy: Immune checkpoint inhibitors**

**Findings:**

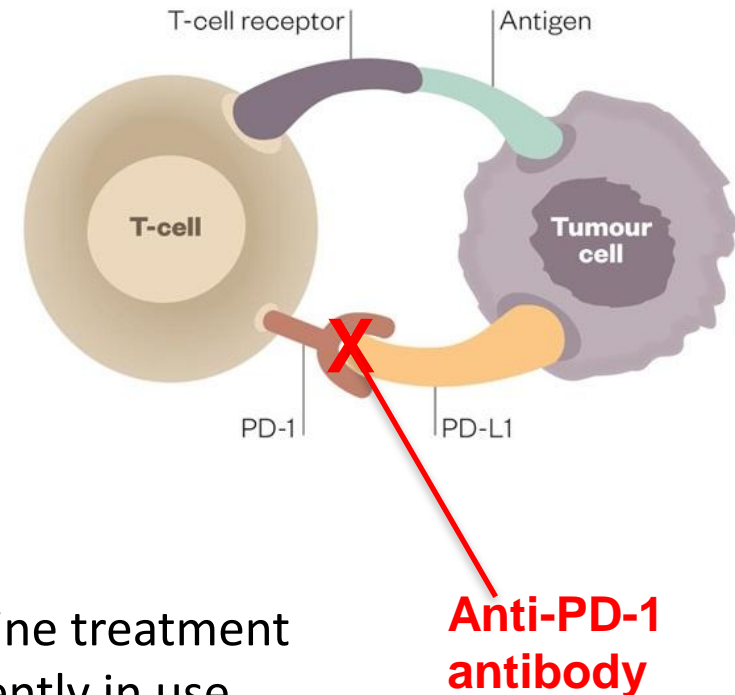
- Two Phase I trials with anti PD-1 antibodies
- Extraordinary response in patients with relapsed disease (50-87%)
- Well tolerated

**Why it's important:**

- New therapeutic modality with potential for 1<sup>st</sup> line treatment
- Safety profile may be superior to cytotoxics currently in use
- Utility for other blood cancer types

**How did LLS help?**

- LLS funded investigators who found very high expression of PD-1 in HL
- Multiple new grant awards in progress to expand utility to other lymphomas



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