LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

someday is today®

# Report to the Nation on Blood Cancer

Leading The Way To Cancer Cures

2017


**Molly Gosch** was just 5 years old when she was diagnosed with acute lymphoblastic leukemia (ALL), the most common form of childhood leukemia. Today, she is 8 years old.

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SECTION 1



# Letter from the President & CEO

It is never a good time to get cancer, but it is a phenomenal time to be fighting it. I can make that statement confidently because, as the president and CEO of The Leukemia & Lymphoma Society, I see the extraordinary progress we are making in fighting blood cancers and helping patients to access lifesaving treatments and cures.

The Leukemia & Lymphoma Society (LLS) developed this *Report to the Nation on Blood Cancer: Leading the Way to Cancer Cures* to educate and engage the public in the fight against blood cancers, which are the third leading cause of cancer deaths among Americans. LLS is the leading global organization dedicated to finding blood cancer cures and advancing access to treatment. We are uniquely able to report on the many advances and accomplishments that have occurred since our founding in 1949. From cutting-edge research and precision medicine innovations to legislative victories that improve access to therapies for cancer patients, LLS plays a leading – and often pioneering – role in the fight against blood cancers.

**"This report is designed to educate the public about blood cancers – where we are now and where we hope to be – so we can continue to advance research and ensure access to treatments to help save more lives."**

Advancing blood cancer treatments and cures also means advancing the science and treatment of other types of cancers and certain chronic diseases. Since 2000, approximately 40 percent of all the newly U.S. Food & Drug Administration (FDA) approved cancer drugs were for blood cancer, and some are now used to treat other forms of cancer and non-malignant diseases. A "win" for blood cancers, therefore, is a "win" for the cancer community overall.

As there are very limited means of preventing blood cancers, the LLS research agenda is focused on finding treatments and cures. In fact, we have invested more than $1 billion in research since our inception 68 years ago.

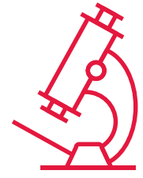
This is an extremely exciting time in the field of blood cancer research and treatment. In the past three years alone there has been remarkable progress in treatments for patients with multiple myeloma (MM) and chronic lymphocytic leukemia (CLL). At the same time, emerging approaches in immunotherapy and precision medicine are showing great promise.

Voluntary health agencies and patient advocacy groups are recognizing now more than ever the key role that they can play in bringing together the entire healthcare "ecosystem" around the development of new therapies for patients. Organizations like LLS are uniquely suited to serve as a convener of academic researchers, regulatory agencies, payers, patients, and the pharmaceutical industry in a way that promotes collaboration and yields new therapies for patients more quickly.

Given the extraordinary progress that the blood cancer community has seen, it would be easy to assume that we are near the goal line of a world without blood cancers.

Unfortunately, this is not the case. Despite our great successes, more than one-third of blood cancer patients still do not survive five years after their diagnosis. The death rate from certain blood cancers, such as AML, remains stubbornly high, with treatment protocols that have not changed in decades. Clearly, much work still needs to be done to understand the genetic underpinnings of blood cancers and find new ways to correct or block those defects.

This report is designed to educate the public about blood cancers – where we are now and where we hope to be – so we can continue to advance research and ensure access to treatments to help save more lives. Public awareness of blood cancer and engagement with research and advocacy organizations like LLS is essential to making sure that we seize this unprecedented time in science to bring advances to blood cancer patients quickly.



We have invested more than

**$1 Billion**

in research since our inception 68 years ago.

At LLS, we have a saying: We are saving lives not someday, but today. With the public's support, and continued collaboration among researchers, doctors, advocacy organizations, pharmaceutical companies, regulators and patients, we are well on our way to making someday today.

Louis J. DeGennaro, PhD
*President and CEO*
The Leukemia & Lymphoma Society

SECTION 2

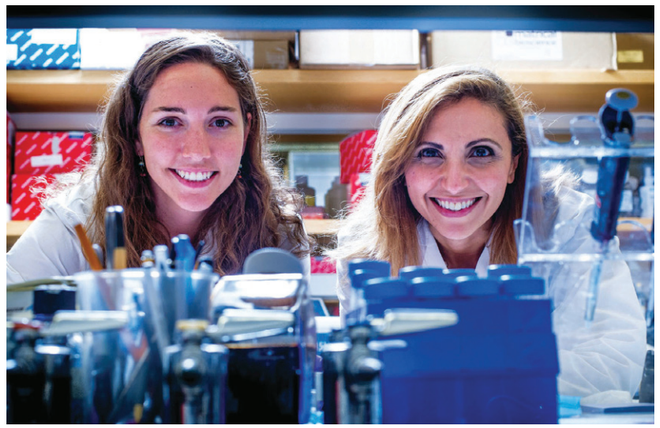


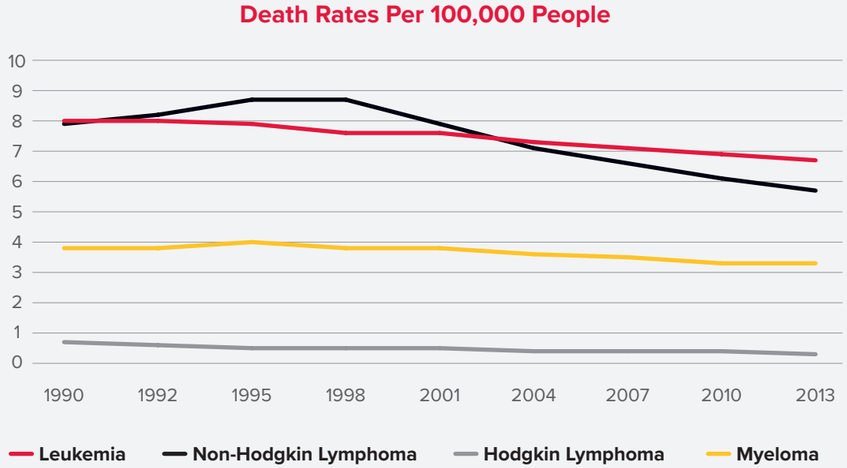
# Our Impact

At LLS, our mission is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families. Compared to any other blood cancer nonprofit, LLS is the largest funder of cutting-edge research and cures.

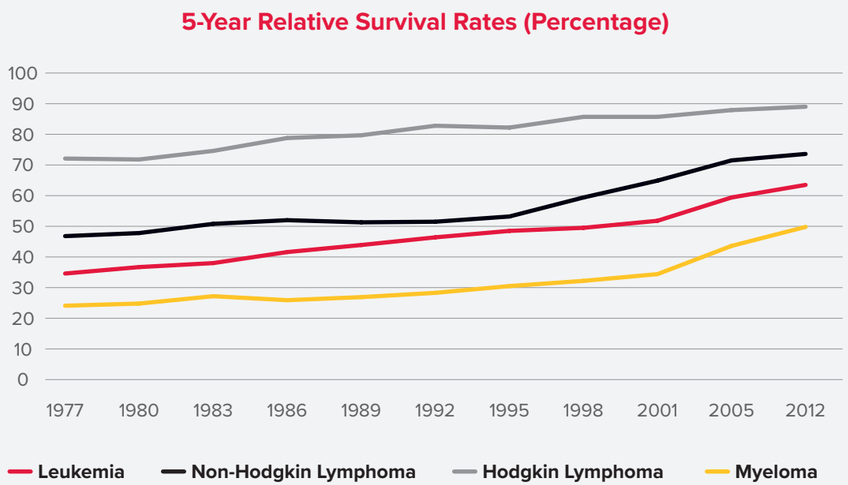
**Our mission is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families.**

Though LLS is known for funding ground-breaking research to find better treatments and cures, we do so much more. We provide free information, education and support services for those who have been impacted by blood cancer. We fight for lifesaving policy changes at the state and federal level to ensure access to quality, affordable, coor-dinated care. We are committed to working tirelessly toward our mission every single day, until we find a cure.






**We've seen an average decline of 20 percent in blood cancer death rates since the 1990s.**




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

## 1 RESEARCH

Since 1949, LLS has supported remarkable scientists whose work has led to breakthrough advances in blood cancer treatments. To date, LLS has invested more than $1 billion in cutting-edge research, funding nearly all of today's most promising advances, and bringing us closer to cures.

We have invested more than

**$1 Billion**

**in cancer research** since 1949.

**4,000**

**research projects** have been supported since 1949.

**300**

**research projects** are being supported at any given time.

Currently funding research at nearly

**100** **medical institutions** across the globe.

**$50–$70 Million**

has been **invested annually** over the past decade.



## 2 EDUCATION & SUPPORT

As the leading source of free blood cancer information, education and support for patients, survivors, families and healthcare professionals, LLS helps patients navigate their cancer treatments and ensures they have access to quality, affordable and coordinated care.

Nearly

**2,000**

**inquiries** per month come in to LLS's Information Specialists from people seeking support.

More than

**$323 million**

**distributed** in co-pay financial assistance

supported more than

**74,600**

**patients** since inception.

More than

**1,000**

**clinical trial searches** were conducted for patients in 2016.

More than

**700**

**connections** were made in 2016 between patients and volunteers diagnosed with the same disease through LLS's Patti Robinson Kaufmann First Connection Program.

More than

**600,000**

**educational booklets** about specific diseases were distributed last year.

More than

**4,000**

patients and caregivers over the past year **joined LLS Community**, an online social network that provides education and support.

Nearly

**200** **family support groups** across the United States.

Our **Information Specialists** are master's level oncology social workers, nurses and health educators who work one-on-one with blood cancer patients and caregivers at no cost to provide information and support tailored to their specific diagnosis and needs. Services include clinical trial searches, financial and emotional support, and up-to-date disease and treatment information. **To reach an Information Specialist, call (800) 955-4572.**

# 3 ADVOCACY

Through our nationwide grassroots network of 104,000 volunteers, LLS advocates for policies at the state and federal level, and is committed to removing barriers to care for blood cancer patients.

**Advanced laws in** **43** **states and Washington, D.C.,** to ensure patients taking oral medications at home receive equitable coverage to patients treated in a clinic.

**Helped pass the** **21st Century Cures Act** into law, ensuring reform that will enable the FDA to speed the review and approval of new therapies.

More than **30,000** **letters** were sent to members of Congress.

**100s** **of calls** were made to House leaders.

**13,000** **advocates** contributed to the passage of 21st Century Cures Act.

**Advocated to ensure proposed federal health-care legislation provides stable, quality, affordable coverage to the thousands of blood cancer patients impacted.**

More than **19,000** **letters** sent **and hundreds of calls** made to members of Congress.

**RESEARCH**

**EDUCATION & SUPPORT**

**ADVOCACY**

# VOLUNTEER LED, STAFF DRIVEN

LLS volunteers selflessly dedicate time and energy to our organization. With countless ways to engage, participate, and volunteer, from individual fundraisers to corporate teams to patient support and advocacy, our volunteers are the drivers behind changing the landscape of cancer.

LLS has more than **15.5 million** **volunteers** across the nation.

**5** **signature campaigns** for volunteers to join: Team In Training®, Light The Night®, Student Series, Man & Woman of the Year™, and Leukemia Cup Regatta®.

More than **27,000** **schools** across the country are donating change in our Student Series campaign.

**150** **Light The Night walks** will be held across the country this year.

Nearly **104,000** **volunteer advocates** are affecting change by advocating for policy at the state and federal level.

Nearly **8,000** **volunteers** across the nation work with our patient services team to provide information and support to patients and caregivers in their communities.

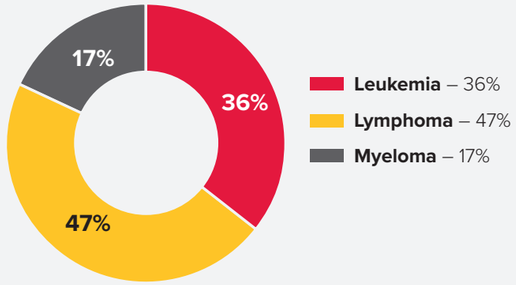
SECTION
3



# What Are Blood Cancers?

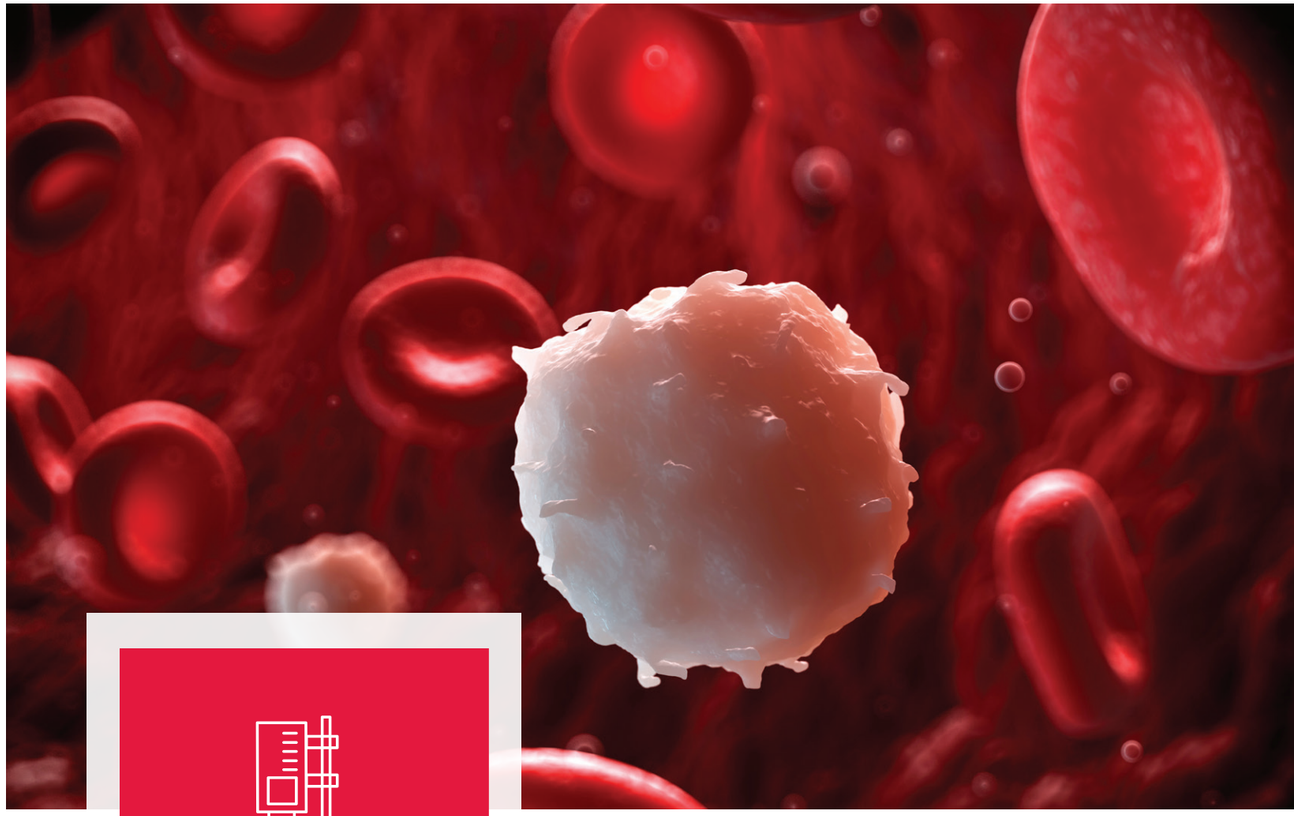
Approximately every three minutes, a person in the United States is diagnosed with a blood cancer. Every nine minutes, someone dies from it. An estimated 173,000 Americans will be diagnosed with leukemia, lymphoma or myeloma in 2017, and more than 58,000 Americans will die from these diseases.

**Estimated New Cases (%) of Leukemia, Lymphoma and Myeloma**




New cases of these blood cancers are expected to account for more than 10 percent of the estimated 1.6 million new cancer cases diagnosed in the U.S. in 2017. Additionally, nearly 1.3 million people in the U.S. are either living with, or are in remission from, leukemia, lymphoma or myeloma.

As the name suggests, blood cancers affect the production and function of blood cells; most of these cancers start in the bone marrow where blood cells are produced. In cancer, the normal blood cell development process is interrupted by uncontrolled growth of an abnormal type of blood cell. These abnormal cells prevent the body from performing many of its functions, such as strengthening the immune system and preventing serious bleeding.



**There are three main types of blood cancers: leukemia, lymphoma and myeloma. In addition, there are other types that affect the blood and bone marrow, including myelodysplastic syndromes and myeloproliferative neoplasms.**

## Blood Cancer Types

There are three main types of blood cancers: leukemia, lymphoma and myeloma. In addition, there are other types that affect the blood and bone marrow, including myelodysplastic syndromes and myeloproliferative neoplasms.

While there are three main types of blood cancer, each cancer is unique and there are many different subtypes. This is why new developments in precision medicine treatments are targeting cancers at the molecular level to ensure patients receive the right treatment at the right time.

## BLOOD CANCER TYPES: LEUKEMIA

# Leukemia

Leukemia begins in a cell in the bone marrow. Once the marrow cell undergoes a leukemic change, the leukemia cells may grow and survive better than normal cells. Over time, the leukemia cells crowd out or suppress the development of normal cells. In 2017, more than 62,000 people will be diagnosed with leukemia, and there are an estimated 364,000 people living with, or in remission from, leukemia in the U.S.

Without a normal number of healthy blood cells, an individual can develop a variety of serious health conditions:

- *Anemia* is characterized by a low number of red cells in the blood, which can cause fatigue and shortness of breath.
- *Neutropenia* is characterized by a low number of white cells, which prevents the immune system from effectively guarding against infection due to a lack of neutrophils (a type of white cell).
- *Thrombocytopenia* occurs when there is a low number of platelets, which can cause bleeding and easy bruising with no apparent cause.
- *Pancytopenia* occurs when there are low numbers of all three blood cell counts: red blood cells, white blood cells and platelets.

The rate at which leukemia progresses and how the cells replace the normal blood and marrow cells is different with each type of leukemia. There are four main types of leukemia (see charts starting on next page).

*Left:* **Isabel Munson** was an 18-year-old college freshman when she was diagnosed with chronic myeloid leukemia (CML). Today, she is a 22-year-old writer, researcher, producer and DJ.

*Right:* In 2015, **Matt Fontanesi** was 33 years old and on his honeymoon with his wife, Dani, when he was diagnosed with acute myeloid leukemia (AML). Today, Matt is still undergoing treatment for transplant-related issues but is doing exceptionally well. The couple lives in San Diego where they volunteer with LLS.



BLOOD CANCER TYPES: LEUKEMIA

## MAIN TYPES OF LEUKEMIA

### 1 Acute Lymphoblastic Leukemia (ALL)

| | |
|---|---|
| **Description** | ALL is a cancer of the bone marrow and blood that affects the immune system. |
| **Prevalence/ New Cases/ Deaths** | 75,300 people are living with ALL. 5,970 new cases are diagnosed each year. 1,440 people die each year. |
| **Typical age at diagnosis** | ALL is the most common cancer found in children and young adults under 20 years of age. About four in ten cases of ALL are in adults. |
| **5-year survival rate** | 70.7% overall. 92% for children/adolescents younger than 15 years, and 94.1% for children younger than 5 years. Less than 20% for adults over age 60. |
| **Treatment** | Chemotherapy. Stem cell transplantation. Tyrosine kinase inhibitors. Clinical trials. |
| **Risk factors** | For most people who have ALL, there are no obvious reasons why they develop the disease. Researchers have found that more developed countries and higher socioeconomic groups tend to have higher ALL rates, but they have not reached any firm conclusions, which suggests that many factors may be involved. Infants born with Down syndrome are at increased risk. People with certain genetic disorders, such as neurofibromatosis, Klinefelter syndrome, Fanconi anemia, Shwachman syndrome, Bloom syndrome and ataxia telangiectasia, are also at increased risk. |

### 2 Acute Myeloid Leukemia (AML)

| | |
|---|---|
| **Description** | AML is a cancer of the bone marrow and blood that affects cells that are not fully developed. AML develops when the DNA of a developing stem cell in the bone marrow is damaged, which is called an "acquired mutation." |
| **Prevalence/ New Cases/ Deaths** | 48,615 people are living with AML. 21,380 new cases of AML are diagnosed each year. 10,590 people die from AML each year. |
| **Typical age at diagnosis** | AML generally affects adults aged 60 years and older. At least half of patients are older than 65 when diagnosed. |
| **5-year survival rate** | 30.3% for patients 55-64 years old. 12.1% for patients 65-74 years old. |
| **Treatment** | Chemotherapy. Stem cell transplantation. Clinical trials. |
| **Risk factors** | Repeated exposure to the chemical benzene has been identified as a potential risk factor. People with certain genetic disorders, such as Down syndrome, Familial Platelet Disorder, Fanconi anemia, Shwachman syndrome and Diamond-Blackfan syndrome, or who have had past chemotherapy or radiation treatments for other cancers, appear to be more likely to develop AML. |

CONTINUES ➔

## BLOOD CANCER TYPES: LEUKEMIA

### MAIN TYPES OF LEUKEMIA (CONTINUED)

#### 3 Chronic Lymphocytic Leukemia (CLL)

| | |
|---|---|
| **Description** | CLL begins in the bone marrow and is the most common form of leukemia in adults. CLL does not completely interfere with the development of mature red cells, white cells and platelets.<br>It can progress either slowly or quickly, depending on the form it takes. It is generally less severe than acute leukemia. |
| **Prevalence/ New Cases/ Deaths** | 162,374 people are living with CLL. 20,110 new cases are diagnosed each year. 4,660 people die each year. |
| **Typical age at diagnosis** | CLL is more common in people who are 70 years or older. |
| **5-year survival rate** | 85.1% overall |
| **Treatment** | Watch and wait. Single or combination drug therapy. Targeted therapies. Monoclonal antibody therapies. White blood cell growth factors. Radiation therapy. Splenectomy. Clinical trials. |
| **Risk factors** | There are no obvious reasons why people develop CLL. Experts have found that in a small number of cases, first-degree relatives (parents and siblings) of people with CLL are three to four times more likely to develop CLL than people who don't have first-degree relatives with the disease. |

#### 4 Chronic Myeloid Leukemia (CML)

| | |
|---|---|
| **Description** | CML develops when the DNA of a developing stem cell in the bone marrow is damaged. CML does not completely interfere with the development of mature red cells, white cells and platelets. CML is usually diagnosed in its chronic phase when treatment is very effective for patients, and is generally less severe than acute leukemia.<br>People with CML have an abnormal chromosome called the Philadelphia (Ph) chromosome, which leads to the development of a cancer-causing gene (oncogene) called the *BCR-ABL* gene. |
| **Prevalence/ New Cases/ Deaths** | 44,386 people are living with CML. 8,950 new cases are diagnosed each year. 1,080 people die each year. |
| **Typical age at diagnosis** | Most cases of CML occur in adults, with a median age of 64 years old. |
| **5-year survival rate** | 65.9% overall. Today, the 5-year survival rate of those newly diagnosed with CML who participated in clinical trials for imatinib is more than 90 percent. |
| **Treatment** | Tyrosine kinase inhibitors. Stem cell transplantation. Clinical trials. |
| **Risk factors** | Two known risk factors are exposure to very high doses of radiation and high-dose radiation therapy (radiotherapy) used to treat other cancers such as lymphoma. |



## BLOOD CANCER TYPES: LYMPHOMA

# Lymphoma

Lymphoma is the name for a group of blood cancers that develop in the lymphatic system, part of the body's immune system. In 2017, there will be approximately 80,500 newly diagnosed cases of lymphoma. An estimated 816,634 people are living with, or in remission from, lymphoma in the U.S.

The two main types are Hodgkin lymphoma and non-Hodgkin lymphoma (see charts starting on next page).

**Steve McHugh,** was 35 years old when he was diagnosed with non-Hodgkin lymphoma in 2010. Today, he is a survivor and renowned chef and owner of Cured, a restaurant in San Antonio, TX.

**Erica Campbell** was 27 years old when she was diagnosed with Hodgkin lymphoma in 2013. Today, she is a survivor, inspirational speaker and model living in Washington, D.C.

# BLOOD CANCER TYPES: LYMPHOMA

## TYPES OF LYMPHOMA

### 1 Hodgkin Lymphoma (HL)

| | |
|---|---|
| **Description** | HL, formerly known as Hodgkin's disease, is a cancer of the blood and bone marrow and one of the most curable forms of cancer. Hodgkin lymphoma starts when an abnormal change to a white cell (called a lymphocyte) causes it to become a lymphoma cell. Lymphoma cells grow and form masses, usually in the lymph nodes, located throughout the body in the lymphatic system. |
| **Prevalence/ New Cases/ Deaths** | 186,607 people are living with HL. 8,260 new cases are diagnosed each year. 1,070 people die from HL each year. |
| **Typical age at diagnosis** | Most common in young adults in their 20s and early 30s or in adults over age 65. |
| **5-year survival rate** | 88.5% overall. For patients who were diagnosed under the age of 45, the survival rate is 94.3%. |
| **Treatment** | Chemotherapy. Combined modality therapy, which is when two or more types of treatment (e.g., radiation, chemotherapy) are used alternately or at the same time. Immunotherapy. |
| **Risk factors** | There are no obvious reasons why people develop the disease.<br>Patients who have a history of a blood test confirming mononucleosis have a three-fold increased risk of HL compared to the general population.<br>People infected with human T-cell lymphocytotropic virus (HTLV) or human immunodeficiency virus (HIV) also have an increased risk of HL. |

### 2 Non-Hodgkin Lymphoma (NHL)

| | |
|---|---|
| **Description** | NHL is a type of cancer that affects the lymphatic system and generally develops in the lymph nodes and lymphatic tissues, but in some cases, NHL involves bone marrow and blood. NHL is not just a single disease – it is actually a diverse group of blood cancers that share a single characteristic in how they develop. |
| **Prevalence/ New Cases/ Deaths** | 630,027 people are living with NHL. 72,240 new cases are diagnosed each year. 20,140 people die each year. |
| **Typical age at diagnosis** | Most common among adults between 65 and 74 years old. |
| **5-year survival rate** | 72.6% overall |
| **Treatment** | Chemotherapy. Radiation therapy. Monoclonal antibody. Stem cell transplantation. Clinical trials. |
| **Risk factors** | For most people who have NHL, there are no obvious reasons why they develop the disease.<br>Living or working in farming communities and exposure to herbicides and pesticides have been shown to increase the risk of developing NHL.<br>Exposure to bacteria and viruses, especially those that suppress the immune system, has been shown to increase the risk of developing NHL. |



BLOOD CANCER TYPES: LYMPHOMA

## TYPES OF NON-HODGKIN LYMPHOMA

### 1 Diffuse Large B-Cell Lymphoma (DLBCL)

AGGRESSIVE OR FAST GROWING NHL

| | |
|---|---|
| **Description** | DLBCL is the most common NHL subtype. It grows rapidly in the lymph nodes and frequently involves the spleen, liver, bone marrow or other organs.<br>DLBCL development usually starts in lymph nodes in the neck or abdomen and is characterized by masses of large B cells (lymphocytes). |
| **Incidence** | About 30 percent of NHL cases. |
| **Typical age at diagnosis** | It most commonly occurs in middle-aged and older persons. The median age of diagnosis is 65. |
| **Overall Survival** | Five-year survival rate is 60% overall. |
| **Treatment** | Combination chemotherapy. |
| **Risk factors** | People with B-cell–activating autoimmune diseases, hepatitis C virus, first-degree family history of NHL, and greater body mass index (BMI) as a young adult are at increased risk for developing DLBCL. |

### 2 Follicular Lymphoma (FL)

INDOLENT OR SLOW GROWING NHL

| | |
|---|---|
| **Description** | FL is the most common indolent (or slow growing) NHL subtype.<br>Abnormal lymphoma cells are grouped together throughout the lymph node. |
| **Incidence** | About 22 percent of NHL cases. |
| **Typical age at diagnosis** | Most people with FL are age 50 or older at diagnosis. |
| **Overall Survival** | Overall survival is more than 10 years. |
| **Treatment** | Watch-and-wait approach. Radiation therapy. Chemotherapy with rituximab followed by radiation therapy. |
| **Risk factors** | Most FL cells have a specific chromosome abnormality (a translocation between parts of chromosomes 14 and 18) that causes the overexpression of a gene, *BCL-2*, and makes the cells resistant to therapy. |

CONTINUES ➔

## BLOOD CANCER TYPES: LYMPHOMA

### TYPES OF NON-HODGKIN LYMPHOMA

| 3 Marginal Zone Lymphoma (MZL) | |
|---|---|
| INDOLENT OR SLOW GROWING NHL | |
| **Description** | MZL includes several subtypes, each categorized by the type of tissue where the lymphoma forms: outside of the lymph nodes (extranodal or MALT), in the lymph nodes (nodal), and the spleen (splenic).<br>It begins in B-lymphocytes in a part of the lymph tissue called the "marginal zone." The disease tends to remain localized. |
| **Incidence** | About 12 percent of B-cell lymphoma cases are MZL. |
| **Typical age at diagnosis** | Most people with MZL are ages 60-65 years old. |
| **Overall Survival** | Five-year survival rate is about 85% overall for *extranodal (MALT)* MZL.<br>Five-year survival rate is about 60-70% overall for *nodal* MZL.<br>Overall survival is 5-10 years for *splenic* MZL. |
| **Treatment** | Removal of the spleen. Single-agent chemotherapy. Combination chemotherapy. Immunotherapy with rituximab. Rituximab combined with chemotherapy. |
| **Risk factors** | People with systemic lupus erythematosus and Sjögren's syndrome, B-cell activating immune conditions, hepatitis C, peptic ulcers, asthma without other atopic diseases, and a first-degree relative with a hematological malignancy, are at increased risk for developing MZL. |

| 4 Mantle Cell Lymphoma (MCL) | |
|---|---|
| AGGRESSIVE OR FAST GROWING NHL | |
| **Description** | Mantle cell lymphoma (MCL) is generally considered an aggressive type of B-cell non-Hodgkin lymphoma. |
| **Incidence** | About 6 percent of NHL cases. |
| **Typical age at diagnosis** | MCL occurs more frequently in older adults—the average age at diagnosis is the mid-60s. |
| **Overall Survival** | Median overall survival is 3-5 years. |
| **Treatment** | Combination chemotherapy. |
| **Risk factors** | MCL is more often diagnosed in males than in females. |



## BLOOD CANCER TYPES: MULTIPLE MYELOMA

# Multiple Myeloma

Myeloma is a cancer of plasma cells, which are a type of white blood cells (also called plasma B cells). Myeloma develops when a plasma cell is mutated. Healthy plasma cells are part of the immune system and make proteins called antibodies, which help fight infection. The most common form of the disease is called multiple myeloma because the malignant cells form tumors in multiple areas of the body.

New treatments called antibody therapies were approved in the last three years and are likely to increase the overall survival rate for patients diagnosed with myeloma.

| Multiple Myeloma | |
|---|---|
| **Description** | Multiple myeloma is the most common form of the disease, and it affects several different areas of the body. |
| **Prevalence/ New Cases/ Deaths** | 110,345 people are living with multiple myeloma. 30,280 new cases are diagnosed each year. 12,590 people die each year. |
| **Typical age at diagnosis** | Most people who develop myeloma are older than 50 years. Fewer cases occur in people younger than 40. |
| **5-year survival rate** | 50.2% overall |
| **Treatment** | Single or combination drug therapy. Chemotherapy. Targeted therapies. Stem cell transplantation. Radiation therapy. Clinical trials. |
| **Risk factors** | Black Americans are nearly twice as likely to develop myeloma as white Americans. Men are at higher risk than women.<br>People with a history of MGUS (monoclonal gammopathy of unknown significance) have increased risk.<br>New research suggests that obese people have a higher incidence of myeloma. |

## BLOOD CANCER TYPES: MYELODYSPLASTIC SYNDROMES

# Myelodysplastic Syndromes

Myelodysplastic Syndromes (MDS) are a group of diseases of the blood and bone marrow, with varying degrees of severity, treatment needs and life expectancy. MDS begin with a change to a normal stem cell in the marrow. These developing blood cells, called blast cells, die as they approach maturity before they would normally be released into the blood. This results in a lower than normal number of circulating blood cells. Approximately 30 percent of patients diagnosed with MDS are at high risk of their disease converting to acute myeloid leukemia (AML).

| Myelodysplastic Syndromes (MDS) | |
|---|---|
| **Description** | MDS are a group of diseases of the blood and bone marrow. MDS develops when blood cell production in the bone marrow increases with more-than-the-normal number of developing blood cells (called “blast cells”) filling the marrow. |
| **New Cases** | An estimated 15,350 new cases of MDS were diagnosed each year from 2009–2013. |
| **Typical age at diagnosis** | Occurs more often in people over 65 years. |
| **Treatment** | Observation of blood cell counts. Transfusions and iron chelation therapy. Erythropoiesis-stimulating agents (ESAs)/growth factors. Managing infections. Drug therapy. Chemotherapy. Stem cell transplantation. Clinical trials. |
| **Risk factors** | Primary MDS – no obvious cause in most patients; repeated exposure to chemical benzene.<br>Treatment-related MDS – previous treatment of chemotherapy and radiotherapy for other cancers. |



## BLOOD CANCER TYPES: MYELOPROLIFERATIVE NEOPLASMS

# Myeloproliferative Neoplasms

Myeloproliferative Neoplasms (MPNs) are types of blood cancers in which bone marrow cells proliferate abnormally and often genetic mutations are found.

| Myeloproliferative Neoplasms (MPNs) | |
|---|---|
| **Types** | There are three types of MPNs: Essential Thrombocythemia (ET), Myelofibrosis (MF) and Polycythemia Vera (PV). |
| **Description** | ET/MF/PV are rare cancers in which the bone marrow cells function abnormally. |
| **New Cases** | About 2.2 out of every 100,000 people are diagnosed with ET each year. About 1.5 out of every 100,000 people are diagnosed with MF each year. About 2.8 out of every 100,000 men and 1.3 out of every 100,000 women are diagnosed with PV each year. |
| **Typical age at diagnosis** | MPNs are usually diagnosed in adult men and women. ET occasionally occurs in older children. |
| **Survival rate** | PV and ET can be managed effectively for a long time and, with proper treatment, people can have a normal or near-normal quality of life. While the median survival for people with MF is about 5 years, people younger than 55 with good prognostic factors have a median survival of 11 years. |
| **Treatment** | Drug therapy. Chemotherapy. Plateletpheresis. Phlebotomy. Stem cell transplantation. Clinical trial. |
| **Risk factors** | For most people, there are no obvious reasons why they develop ET. About 90% of people with MF have a mutation in one of three genes – *JAK2*, *CALR* or *MPL*. Almost all people with PV have a mutation of the *JAK2* gene. |

SECTION
4

# Major Accomplishments in Blood Cancer Treatment and Survival: 1949 – Today

Since the founding of LLS (originally known as The Leukemia Society of America) in 1949, we have made enormous strides in our understanding and treatment of blood cancers.

LLS has supported the development of some of the most effective and widely used therapies, from the early days of combination chemotherapies and bone marrow transplants more than 50 years ago, to immunotherapies and precision medicine today. Our support – funding both clinical and basic science – has led to groundbreaking clinical trials and breakthrough research on effective treatments for blood cancer patients.

**LLS has played a major role in bringing groundbreaking treatments to blood cancer patients.**

LLS has played a major role in bringing groundbreaking treatments to blood cancer patients.

Through LLS's numerous research programs and work to improve patient access to better treatments, survival rates for many blood cancer patients have doubled, tripled and even quadrupled since 1960. LLS is especially proud that some of the therapies first approved for blood cancer patients are now helping patients with other types of cancers and serious diseases.



**CML SPOTLIGHT**

## From Fatal Disease to Manageable Condition

Imatinib (Gleevec®) is a targeted therapy originally approved by the FDA in 2001 for the treatment of chronic myeloid leukemia (CML), turning a once fatal diagnosis into a manageable condition for most patients. Given its remarkable success, the drug also has been approved by the FDA to treat other blood cancers, including Philadelphia Positive (PH+) acute lymphoblastic leukemia (ALL) in children, rare stomach cancers and skin cancers.

**Brian Druker, MD,** Director of The Knight Cancer Institute at Oregon Health & Science University

The journey to develop imatinib took more than a decade, led by the extraordinary efforts of Brian Druker, MD, who is now the Director of The Knight Cancer Institute at Oregon Health & Science University (OHSU). Druker found a way to "turn off" the enzymes that cause cancer. Researchers around the country – and the world – were involved in different aspects of the research and development that led to the discovery of imatinib. Funding from LLS provided critical support at key progress points, including the discovery of a genetic abnormality by LLS-funded investigator Janet Rowley, MD, of the University of Chicago, as well as proof of concept work by Druker.

Clinical trials of the drug began in 1998 and the results were astonishing: 98% of patients with CML showed dramatic improvements. Today, the five-year survival rate of those newly diagnosed with CML who participated in clinical trials for imatinib is more than 90 percent. The overall survival rate for CML patients is 63 percent. While the reasons for this disparity are not well understood, one factor may be that patients in clinical trials receive continuous care and do not incur out-of-pocket costs, resulting in better adherence to treatment.

Imatinib is also being tested in studies for patients with a type of colon cancer, neurofibromatosis, and diabetes. Imatinib works by inhibiting a group of enzymes that serve many functions, including roles in cell growth and proliferation, as well as the autoimmune response in diseases such as diabetes.

A TIMELINE

# The impact of LLS funding over decades in the fight against cancer

**In 1964, the five-year survival rate for children with the most commonly diagnosed pediatric leukemia, ALL, was 3 percent. Today, it's approximately 90 percent.**

## 1949

In 1949, Rudolph and Antoinette Roesler de Villiers, who lost their teenage son, **Robert**, to leukemia in 1944, established the first incarnation of what became The Leukemia & Lymphoma Society. The impact was felt right away and the 1950s and 1960s saw some major treatment advances that were revolutionary for the time.

## 1950

**George H. Hitchings, PhD**, and **Gertrude B. Elion, D.Sc.**, began collaborating in 1945 and developed the most widely used anti-leukemia drugs in 1950-1951. Both later served as medical and scientific advisors to LLS, and earned the 1988 Nobel Prize in Physiology and Medicine.

Photo: Wellcome Library, London

## 1955

In 1955, **William Dameshek, MD**, became a medical and scientific advisor to LLS, organizing its grant review process. In 1946, he was a lead investigator of studies that led to the first anti-cancer chemotherapy.

## 1955

**James Holland, MD**, was among the first LLS grant recipients, receiving funding in 1955; he went on to become one of the first researchers to advance combination chemotherapy.

Photo: PBS/*Cancer: The Emperor of All Maladies*

## 1956

**E. Donnall Thomas, MD**, conducted the first successful bone marrow transplant on a leukemia patient in 1956. Thomas was an LLS advisor in the 1960s, and was awarded the 1990 Nobel Prize in Physiology and Medicine.

Photo: Susie Fitzhugh

## 1965

The first combination chemotherapy was developed for childhood leukemia patients by **Emil "Tom" Frei, MD** (background), and Emil J. Freireich, MD, under the leadership of **Gordon Zubrod, MD,** (foreground), Director of the National Cancer Institute's Clinical Center.

Photo: The Zubrod Family

## 1970s

The advent of the 1970s brought an early understanding of the genetics of cancer with the discovery of oncogenes.

For his role in identifying oncogenes, **J. Michael Bishop, MD**, an advisor to LLS, later received the 1989 Nobel Prize in Physiology and Medicine. By the 1980s, researchers advanced this knowledge further.

Photo: PBS/*Cancer: The Emperor of All Maladies*

## 1980

**Riccardo Dalla-Favera, MD**, and his LLS-funded research team studied the molecular pathway involved in immune B-cell activation and how those pathways became dysregulated in B-cell cancers. A few decades later, he led a team of LLS-funded researchers investigating the genetic origins of B-cell non-Hodgkin lymphoma.

## 1985

In 1985 and again in 1989, **Hagop Kantarjian, MD,** received LLS scholar awards to study new approaches to treating patients with chronic myeloid leukemia (CML). He later played a significant role in the development of the first targeted therapy to treat CML patients.

*"LLS is where it is today because it has groomed a remarkable generation of scientists and physician scientists who have led the extraordinary advances in treatment of hematologic malignancies."*

**GARY GILLILAND, MD, PHD, PRESIDENT OF FRED HUTCHISON COMPREHENSIVE CANCER CENTER**

## 1990

By the 1990s, the discovery of genetic pathways was followed by the first FDA approvals for targeted blood cancer drugs.

In 1990, LLS-funded researcher **Susan Rabinowe, MD**, and others, showed the protein *CD20* is consistently present on B-cell chronic lymphocytic leukemia (CLL) cells.

## 1996

In 1996, LLS-funded investigator **Brian Druker, MD**, tested a *BCR-ABL*-blocking drug. This resulted in the first clinical trial, led by Druker and Charles Sawyers, MD, of *STI-571*, later known as imatinib (Gleevec®) in 1998.

## 1996

**Charles Sawyers, MD**, began studies that helped identify the genetic causes of resistance to imatinib, leading the way to the development of follow-on therapies for patients with imatinib-resistant CML.

## 2001

From 2001 to 2005, LLS-funded researchers David Maloney, MD, **Margaret Shipp, MD**, Felipe Samaniego, MD, and others showed rituximab (Rituxan®) improved the effectiveness of the standard chemotherapy regimen for diffuse large B-cell lymphoma patients.

## 2004

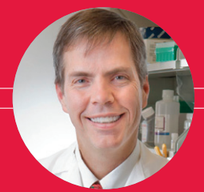
A multi-institution Phase II trial in 2004, led by **Alan List, MD**, and clinical colleagues, including LLS-funded investigator Richard Stone, MD, confirmed the efficacy of lenalidomide (Revlimid®) for patients with a rare form of myelodysplastic syndromes (del(5q) MDS).



**Forty years ago, the five-year survival rate for someone diagnosed with myeloma was 10 percent. Today, it's about 50 percent. Over the past decade, treatment options, many supported with LLS funding, have increased significantly, and the survival rates are expected to continue to increase.**

## 2010

**Stephan Grupp, MD, PhD**, played an instrumental role in the advancement of immunotherapy, particularly in treating pediatric acute lymphoblastic leukemia (ALL) patients. Almost two decades before, LLS awarded Grupp with a Career Development Special Fellow Award, which he credited with helping to launch his career.



## 2012

LLS-funded research continued to drive breakthroughs and advances, transforming cancer's grim past into a promising future. From 2010-2012, **John Byrd, MD,** and colleagues published Phase II trial results for ibrutinib in CLL and small lymphocytic lymphoma (SLL) patients, showing safety and lasting remissions.

## 2014

New advances in immunotherapy include an approach called immune checkpoint inhibitors, that unleash the immune system by removing the "brakes." This approach is advanced by researchers, including LLS-funded investigators Margaret Shipp, MD, and **Steve Ansell, MD, PhD**.

## 2016

In 2016, FDA approved of a new therapy, venetoclax (Venclexta®), for patients with a high-risk form of chronic lymphocytic leukemia. Since 2002, LLS continuously supported research by Jerry Adams, PhD, **Andrew Roberts, MBBS, PhD**, and colleagues to advance this therapy.

## Today

LLS is supporting emerging work to understand how mutations lead to blood cancer in order to help prevent it. Today, LLS supports the work of Benjamin Ebert, MD, PhD, and **Irene Ghobrial, MD**, who are studying how to prevent recurrence after treatment as well as precursor diseases that lead to blood cancer.

CLL SPOTLIGHT

# Targeted Therapy Extends Survival and Improves Quality of Life

Ibrutinib (Imbruvica®) is a therapy that targets the Bruton's tyrosine kinase (BTK) enzyme, a promoter of B-cell cancers, including chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL), Waldenstrom's macroglobulinemia (WM), follicular lymphoma (FL), diffuse large B-cell lymphoma (DLBCL), hairy cell leukemia (HCL) and multiple myeloma (MM).

John C. Byrd, MD, of The Ohio State University – a long-time LLS grant recipient – began his research to tackle CLL by targeting BTK with ibrutinib in 2009. Because CLL does not have a single genetic mutation, many experts predicted that precision medicine would not be an effective approach. However, clinical trials of ibrutinib showed unprecedented response rates.


**John C. Byrd, MD**, of The Ohio State University

First approved in 2013 for mantle cell lymphoma, ibrutinib was then approved for patients with relapsed or refractory CLL in 2014, with additional approvals following in 2015 and 2016. For thousands of patients, this drug has not only extended their survival, but also improved their quality of life.

In 2016, a study by an LLS Specialized Center of Research (SCOR) team led by Tom Kipps, MD, UCSD, showed ibrutinib to be superior to standard therapy with chemotherapy, leading the FDA to approve it as a front-line treatment for CLL patients.



CLL SPOTLIGHT

# A New Option for Patients Not Responding to Treatment

Venetoclax (Venclexta ®) is a targeted therapy approved by the FDA in April 2016 to treat patients with a high-risk form of chronic lymphocytic leukemia (CLL) who have not responded to at least one other therapy. The oral medication is for patients who have a rare subset of CLL known as 17p deletion, which means they are missing a piece of chromosome 17.

Approximately 30 percent of CLL patients who do not respond to therapy or who have relapsed have the 17p deletion. Venetoclax works by targeting the B-cell lymphoma 2 (*BCL-2*) protein, which supports cancer cell growth.

Since 2002, LLS has supported more than $15 million in research leading to the development of this drug through its collaborative Specialized Center of Research (SCOR) grant program. Jerry Adams, PhD, of the Walter and Eliza Hall Institute of Medical Research in Melbourne, Australia, leads the research team. Andrew Roberts, MBBS, PhD, a member of Adams's SCOR team, was one of the clinicians who led studies of the pivotal Phase 2 clinical trial of venetoclax, leading the FDA to approve the therapy.

LLS is currently supporting research to investigate venetoclax as a treatment for patients with acute lymphoblastic leukemia (ALL), mantle cell lymphoma (MCL) and acute myeloid leukemia (AML). This includes the work of Anthony Letai, MD, PhD, Dana-Farber Cancer Institute, who is studying the effectiveness for AML patients, with encouraging results reported to date.


Photo: Walter and Eliza Hall Institute of Medical Research in Melbourne, Australia

**Jerry Adams, PhD,** of Walter and Eliza Hall Institute of Medical Research in Melbourne, Australia

SECTION
5



# Accelerating Treatments Through Innovative Research

There has been tremendous momentum and excitement in blood cancer research over the past several years as precision medicine and immunotherapies such as CAR-T cell therapy have shown very promising early results in treating patients and extending lives.

**Our growing understanding of the genetic underpinnings of blood cancer and the pace of drug discovery have been super-charged.**

From the discovery of imatinib (Gleevec®), which has transformed chronic myeloid leukemia (CML) from a devastating disease to a chronic condition, to new monoclonal antibody treatments for myeloma, to immune checkpoint inhibitors, our growing understanding of the genetic underpinnings of blood cancer and the pace of drug discovery have been super-charged.

LLS supports the full spectrum of research from bench to bedside – that is, from basic, laboratory-based research to large-scale clinical trials – with the singular goal of accelerating treatments and cures to the more than 1.2 million people in the United States living with some form of blood cancer.

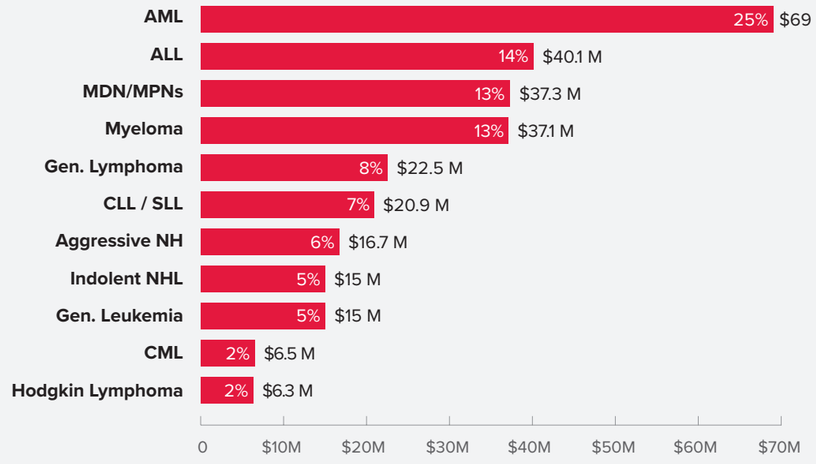
LLS supports multiple research programs through specialized grants, collaborations and venture philanthropy. Our Career Development Program is designed to support promising investigators in their developing careers. Specialized Center of Research (SCOR) grants fund multinational, multi-disciplinary teams of researchers who are engaged in collaborative efforts, while our Translational Research Program (TRP) brings promising research findings from the laboratory to clinical development. Our New Idea Awards fund innovative approaches that may fundamentally change the understanding,

**LLS supports the full spectrum of research from bench to bedside – that is, from basic, laboratory-based research to large-scale clinical trials.**

diagnosis and/or treatment of blood cancers and related pre-malignant conditions. Through our Therapy Acceleration Program® (TAP), we partner directly with academic institutions and biotechnology companies to help accelerate the development of promising therapies.

## Our Investment

THE AMOUNT LLS IS CURRENTLY COMMITTED TO INVESTING IN RESEARCH



# OUR RESEARCH PORTFOLIO

## 1 INVESTS IN YOUNG SCIENTISTS

**Career Development Program** attracts and retains the highest quality young scientists, launching the careers of many of the most productive clinicians and researchers in cancer.

Nearly **3,000** grants awarded since 1953

More than **$370 million** **invested in grants**

at more than **400** medical and academic institutions

**THIS INCLUDES:**

**3** **Nobel Laureates**

More than **15** **members** of the National Academy of Science

**9** **directors** of comprehensive cancer centers

**10** department chairs/section directors

## 2 LEADS THE CHARGE TO BEAT AML

**Beat AML Master Trial®** is a groundbreaking, collaborative clinical trial for acute myeloid leukemia (AML), a deadly disease that has seen few improvements in treatments in more than 40 years.

**11** **cancer centers** will participate by summer 2017

**7** or more **treatment study arms** will open by summer 2017

**60** is the age that newly diagnosed patients can enroll in trial

Patient's genetic analysis completed within **7 days**

## 3 TRANSLATES RESEARCH FROM BENCH TO BEDSIDE

**Translational Research Program** was developed in 1996 to provide early-stage support for clinically translatable research in blood cancers.

LLS has awarded approximately **700** **grants** through this program

investing **$313 million**

# OUR RESEARCH PORTFOLIO

## 4 FAST-TRACKS TREATMENT TO PATIENTS

**Therapy Acceleration Program**® expedites getting treatments to patients by supporting promising projects and clinical trials through collaborations with biotechnology companies.

More than **$100 million** invested in **50** projects since 2007.

More than **$700 million** invested by **8** companies to push TAP closer to the finish line.

Approximately **$10 million** invested per year

**17 projects** currently in the pipeline

## 5 FOSTERS COLLABORATION ACROSS DISCIPLINES & INSTITUTIONS

**Specialized Center of Research** program brings together established investigators across different disciplines from one or several institutions to develop a research program over five years. These synergistic collaborations greatly advance research progress and clinical applications.

Since the program started in 2000, **$300 million** has been awarded across **50 awards** and **30 recipients**

## 6 ENCOURAGES "OUT OF THE BOX" THINKING

**New Idea Award** supports innovative "out of the box" approaches that may fundamentally change the understanding, diagnosis and/or treatment of blood cancers, but may not be candidates for conventional government funding.

Since the program started in 2013, LLS has awarded approximately **25 grants** investing **$2.25 million**

SECTION 6

# Our Priorities for Blood Cancer Cures

The following are some of our priority areas we've set for our agenda in the coming years:

**In the past five years alone, LLS has invested about $100 million in AML research, with a focus on understanding the underlying causes of the disease to develop better therapies and save more lives.**

## 1. Using Precision Medicine to Beat AML

In 2013, LLS launched an unprecedented attack against acute myeloid leukemia (AML), a deadly disease which has seen little to no improvement in treatments in more than 40 years.

AML is among the most lethal of the blood cancers, responsible for more than 10,500 deaths each year. The standard of treatment for AML – a combination of toxic chemotherapies – has remained the same for more than four decades. Overall prognosis remains poor, with a five-year survival rate below 20 percent for patients over age 60.



LLS teamed up with Brian Druker, MD, and his research team at The Knight Cancer Institute at Oregon Health & Science University (OHSU) to lead the first phase of Beat AML. This initial phase deployed advanced genomic technology to create a profile of genetic defects in AML cells.

The goal was to collect 900 samples from AML patients and screen more than 37 proprietary novel drugs and combinations. To date, more than 700 samples have been collected. This ongoing work is helping lay the groundwork for the second phase of Beat AML, a Master Trial that is testing multiple drugs at multiple sites to find the most effective treatments based on individual patients' specific genetic profiles.

LLS launched the Beat AML Master Trial® in October 2016. it is the first collaborative precision medicine trial in a blood cancer. This groundbreaking clinical trial is using advanced genomic technology to identify patients' genetic mutations and test several investigational, targeted drugs to treat those patients. This trial involves multiple medical institutions, drug companies and the FDA, all of whom have committed to working collaboratively to drive this master clinical trial forward. LLS hopes it will serve as a model for other cancer research and discovery programs.

With support and guidance from the FDA, and LLS as the sponsor, the ambitious Beat AML Master Trial® seeks to change the paradigm for how this deadly cancer is treated. LLS is

### Beat AML Master Trial®

uniquely qualified to lead this unprecedented clinical trial collaboration, which is a rare role for a nonprofit organization and a first for LLS.

Three world-renowned blood cancer scientists lead the clinical trial: Brian Druker, MD, The Knight Cancer Institute at Oregon Health & Science University (OHSU); John Byrd, MD, The Ohio State University Comprehensive Cancer Center; and Ross Levine, MD, Memorial Sloan Kettering Cancer Center. Many other prominent researchers lead each arm of the trial at their institutions. LLS expects up to 500 patients to enroll in the trial. The trial is on target to surpass 40 patients enrolled by June 2017.

Beat AML demonstrates LLS's ability to convene the medical and research communities to think and act boldly in the quest for new and better treatments for blood cancer patients, and supports our goal to accelerate the rate at which precisely targeted breakthrough therapies reach the patients who urgently need them.

Because of the urgent unmet medical need, LLS is taking a multi-pronged approach to find cures for AML. Currently, about one-fourth of our research budget is dedicated to AML. In the past five years alone, LLS has invested about $100 million in AML research, with a focus on understanding the underlying causes of the disease to develop better therapies and save more lives.

Another promising approach for the treatment of AML is a result of a partnership through LLS's Therapy Acceleration Program® (TAP). In 2009, LLS started to collaborate with Celator Pharmaceuticals, which was acquired by Jazz Pharmaceuticals in May 2016. The goal was to advance a therapy called CPX-351 (Vyxeos®), which has performed better than standard therapy in clinical trials for patients with a subtype of AML. In fact, the latest Phase 3 data showed it reduced the risk of death by 31 percent for older patients with high-risk secondary AML. In April 2017, Jazz Pharmaceuticals submitted its application to begin the process of requesting FDA approval of this therapy.

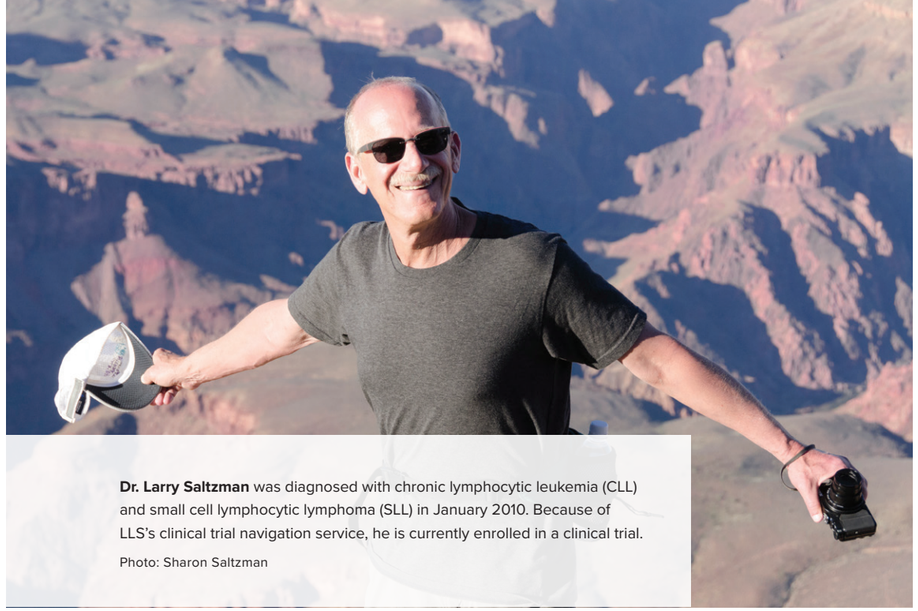
With our ability to convene the landmark Beat AML Master Trial®, combined with a significant investment in scientific research, as well as patient support and education, we are leading the way to accelerate new treatments and cures for this deadly disease.

## 2. Connecting Patients to Clinical Trials

One of LLS's most important roles in accelerating the development of new therapies is connecting cancer patients with clinical trials, which are the essential foundation for the advancement of scientific research and life-saving treatments. Despite the public's recognition of their value, many clinical trials are unable to achieve their goals because they cannot enroll enough patients. In fact, less than 5 percent of cancer patients actually enroll in clinical trials focused on finding cancer treatments.

There are many reasons for the low clinical trial enrollment rate, including patients' misperceptions about clinical trials, and financial and logistical concerns. Provider attitudes and beliefs about trials are also a factor; many providers do not feel comfortable discussing trials with their patients and view clinical trials only as an option of last resort.

LLS has increased its efforts to help patients enroll in clinical trials by expanding our personalized clinical trial navigation services, aiming to help more than 1,000 patients enroll in clinical trials over the next five years. Our clinical trial support service matches a patient's unique clinical, social and financial situation to available clinical trials.




**Dr. Larry Saltzman** was diagnosed with chronic lymphocytic leukemia (CLL) and small cell lymphocytic lymphoma (SLL) in January 2010. Because of LLS's clinical trial navigation service, he is currently enrolled in a clinical trial.

Photo: Sharon Saltzman

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Medical experts in LLS's Clinical Trial Support Center (CTSC) help patients to find and enroll in clinical trials based on highly detailed, individualized assessments, along with the provision of in-depth guidance, support and resources. In fact, LLS's medical experts engage in many interactions with each patient and often call clinical trial sites to help with trial enrollment. The result is that the majority of patients working with LLS's CTSC have enrolled in one or more clinical trials.

Our clinical trial specialists work tirelessly to help patients find and enroll in clinical trials to access the most cutting-edge treatments for their diagnosis. These specialists are part of LLS's team of Information Specialists, who are master's level oncology social workers, nurses and health educators. Through a toll-free call center (800-955-4572), they work one-on-one with blood cancer patients and caregivers at no cost to provide support and information.

LLS also collaborates with Dana-Farber Cancer Institute on the Blood Cancer Research Program (BCRP), which is designed to provide access to clinical trials in communities where there are no major medical centers. Through its network of twelve sites across the nation, BCRP aims to accelerate the advancement and expansion of access to well designed and innovative clinical trials for blood cancer patients treated at community sites.

By increasing access to and enrollment in clinical trials, our goal is to provide patients the opportunity to receive the newest treatments and best care, while also helping to accelerate life-saving treatments to help patients in the future.

**In March 2017, LLS launched a program with the National Black Church Initiative (NBCI), a faith-based coalition of churches, to address the striking health disparities among African Americans with myeloma.**

## 3. Increasing Our Investment in Myeloma Cures

In the U.S., myeloma is the second most common blood cancer. While there have been many new therapies approved over the last decade and researchers are studying promising treatments, the disease remains incurable. Only 49.6 percent of patients diagnosed with myeloma will survive five years after diagnosis.

To address this urgent unmet medical need, LLS is taking a multi-pronged approach over the next five years to improve outcomes for patients, by investing in scientific research as well as education and outreach efforts to improve patient access to the most promising, cutting-edge treatments.

In March 2017, LLS launched a program with the National Black Church Initiative (NBCI), a faith-based coalition of churches, to address the striking health disparities among African Americans with myeloma. In fact, black Americans have twice the incidence of myeloma as white Americans, and recent studies show they are significantly less likely to receive the newest treatments or combination therapies, and are more likely to experience treatment delays, including transplant delays.

Over the next five years, through the multifaceted program called *Myeloma Link: Connecting African American Communities to Information, Expert Care, and Support*, LLS will provide education about myeloma and clinical trials to African Americans in major cities throughout the country.

The goal of the initiative is to improve access to novel therapies and quality of life among African Americans with myeloma by providing tools and resources to navigate the treatment landscape more effectively and cope with the disease.

On the research front, LLS plans to significantly increase its current annual investment in myeloma research over the next three to five years, with a focus on resistance to therapy, targeted therapies, immunotherapies and preventing progression of the disease.

A major focus of this research investment is to develop targeted therapies that can be used alone or in combination with other newly approved drugs, as well as immunotherapies that will harness the immune system to fight myeloma.

Through a renewed investment in cutting-edge research and patient education over the next five years, our goal is to find cures and significantly improve the lives of those living with myeloma.



## 4. Driving Immunotherapy Forward

Since its beginnings, LLS has invested in the promising field of immunotherapy, which harnesses a patient's own immune system to kill cancer cells. It remains a major focus of our research commitment today.

In fact, an LLS advisor, E. Donnall Thomas, MD, conducted the first successful bone marrow transplant on a leukemia patient in 1956. Bone marrow transplants provided the first example of how the immune system can help fight cancer.

Today, immunotherapy is one of the most promising treatment approaches for cancer. One groundbreaking approach is called chimeric antigen receptor (CAR) T-cell therapy, which LLS has been investing in since the mid-1990s.

This therapy has had remarkable early results in the treatment of blood cancer, proving to be miraculously effective in some patients with certain types of leukemia, lymphoma and most recently, myeloma. Dozens of adults and children who were once near death are now in remission, and some remain healthy up to five years after treatment.

In this therapy, immune T-cells are removed from the patient's body, and then are genetically engineered to produce a protein on the surface of the T-cells that can bind and recognize the cancer cells. These engineered T-cells are multiplied in the lab and eventually given back to the patient through an intravenous infusion. Once the T-cells "home in" on the cancer cells, this triggers the T-cells to multiply further and kill the cancer-ridden cells.

**Doug Olson** was diagnosed with chronic lymphocytic leukemia at age 49. Today, he is a six-year survivor who benefited from CAR-T immunotherapy.

**In this therapy, immune T-cells are removed from the patient's body, and then are genetically engineered to produce a protein on the surface of the T-cells that can bind and recognize the cancer cells.**

LLS has been funding CAR-T since the beginning, through support of a team led by Carl June, MD, University of Pennsylvania, and his colleagues at Children's Hospital of Philadelphia, who are all credited with being among the pioneers of this therapy. LLS has invested in the work of Dr. June and his colleagues since 1998, investing more than $21 million to advance this treatment.

The University of Pennsylvania's program was later licensed to Novartis. In March 2017, Novartis filed for FDA approval of this therapy and included clinical trial data showing the therapy resulted in remission of 82 percent of patients after three months.

Last year, LLS announced an additional $11 million investment in immunotherapy. At Memorial Sloan Kettering Cancer Center, LLS's SCOR program is funding a research project aimed to advance a new generation of CAR-T cells to both eradicate cancer cells and shut down cancer's immune defense mechanisms.

**Last year, LLS announced an additional $11 million investment in immunotherapy.**

Through its Therapy Acceleration Program®, LLS collaborates with Kite Pharma, a biotechnology company focused on immunotherapy, to fund this promising therapy through a clinical trial. Positive data from the clinical trial was released showing that more than one-third of refractory aggressive non-Hodgkin lymphoma (NHL) patients in the study showed no signs of the disease after six months. Kite filed for FDA approval of this therapy in March 2017.

While the FDA has not yet approved CAR-T immunotherapy, patients are able to enroll in clinical trials. LLS will continue to invest in this promising therapy, while providing support through our clinical trial navigation service to patients who are either currently in a CAR-T clinical trial or interested in enrolling in one.

## 5. Advocating for Quality, Affordable Care

Even with our focus on drug discovery, LLS recognizes that finding cures is not enough; we need to ensure that patients have access to the treatments they need to live longer, better, healthier lives. We are dedicated to removing barriers to care, and our network of nearly 104,000 advocates has a powerful voice in driving policies that accelerate the development and approval of innovative treatments and ensuring that patients have sustainable access to quality, affordable, coordinated care.

As the voice for all blood cancer patients, LLS's Office of Public Policy has achieved groundbreaking results for patients at both the state and federal level. For example, LLS helped to drive the 21st Century Cures Act forward and advocate for oral parity legislation, which has been signed into law in 43 states and Washington, D.C.

While there has been great progress, much work remains to ensure that federal healthcare rules continue to protect cancer patients. LLS works to advance policies that accomplish the following goals:

- ✓ **Guarantee Access** – Newly diagnosed cancer patients must continue to have the right to purchase quality, affordable health insurance to help them access the care they need.
- ✓ **Ensure Quality** – Policymakers must continue to provide minimum quality standards that protect patients from being locked out of necessary treatment due to barebones coverage.



**LLS recognizes that finding cures is not enough; we need to ensure that patients have access to the treatments they need to live longer, better, healthier lives.**

- ✓ **Promote Affordability** – Premium assistance and cost-sharing limits that allow a cancer patient to use their coverage must be improved.
- ✓ **Provide Stability** – Policymakers must provide cancer patients with the peace of mind that every patient will have access to affordable, quality coverage.

**Our team of advocates also works to remove barriers and ensure patients are able to access cancer treatments. Our goals include:**

- ✓ **Reducing Out-of-Pocket Costs for Medicare Patients** – LLS advocates for protections against prohibitive patient out-of-pocket costs for life-saving prescription medications. LLS supports legislation to allow Medicare Part D beneficiaries to pay a flat, reasonable copay for essential medications with no less costly alternatives. LLS also supports limiting the total annual out-of-pocket expenses for Medicare patients with high treatment costs. In the near future, legislation will be introduced advancing these policies.
- ✓ **Oral Parity for Cancer Drugs** – LLS works to advance federal and state legislation that would promote fairness and innovation by preventing insurance plans from requiring patients to pay significantly more out-of-pocket costs for cancer drugs based on how the drug is administered. A bipartisan coalition of House and Senate champions are drafting legislation to advance this important protection for cancer patients.

**We also focus on accelerating new therapies and cures for patients through the following:**

- ✓ **Patient Engagement in Drug Development & Approval** – LLS advocates for improvements that can speed the development and approval of safe and effective treatment options for blood cancer patients. LLS is working to secure Congressional approval of FDA user fee legislation that will provide the FDA with the resources necessary to fulfill its commitment to amplify the voice of patients throughout the development and approval processes.
- ✓ **Expanded Access Reform** – LLS supports necessary improvements to the FDA's expanded access program, which is intended to help facilitate patient access to investigational drugs when they have no other treatment options. LLS is working to build on transparency reforms enacted in the 21st Century Cures Act that will help patients and providers navigate this complex program.

Our advocacy efforts are unapologetically patients first, and we will continue to advance policy at the state and federal level to ensure patients have access to quality, affordable, coordinated care.

SECTION 7

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

someday is today®

# How the Public Can Help Fight Blood Cancer

As the largest voluntary cancer research agency specifically focused on finding cures and treatments for blood cancer patients, LLS supports hundreds of cancer scientists and research projects around the world. But the fight against blood cancers cannot be won without the public's support.

**Every voice, every action, every contribution is needed and valued. LLS encourages the public to join the fight in one or all of these ways:**

Blood cancer patients, their families, friends and colleagues, along with individuals who have not been touched directly by cancer, are powerful allies in the fight, and their voices and actions carry tremendous influence with researchers, physicians, policymakers, regulators and industry. Yet, we often hear from the public that they are not sure how to get involved and whether their individual involvement "counts." Our answer is an unequivocal "yes." Every voice, every action, every contribution is needed and valued. LLS encourages the public to join the fight in one or all of these ways:



## 1 Learn About and Help LLS Advocate for Policies that Accelerate Treatments and Cures

LLS advocates for public policy positions that accelerate progress toward cures for blood cancers and improve the quality of life of patients, along with their friends and families.

Policy advocacy is essential because critical challenges remain in moving new therapies through the regulatory review process, and many blood cancer patients still face significant barriers to access the care they need. Regulatory agencies and legislative bodies at both the state and federal level play a pivotal role in addressing these problems. To advance our policy goals, LLS encourages the public to contact their elected representatives – in Congress and in state legislatures – to share with them the impact that blood cancers have on millions of Americans each year, and to urge responsible policies that will address the serious burdens of these diseases.

**More information about LLS's policy advocacy efforts, overseen by our Office of Public Policy in Washington, D.C., can be found at www.lls.org/advocate**.

## 2 Encourage Family and Friends with Cancer to Participate in Clinical Trials

Advances in treatment for blood cancers depend on clinical trials of new therapies or new combinations of therapies. Today, virtually all of the established treatments for cancer are available because of clinical trials.

Cancer patients, especially the newly diagnosed, should be encouraged to talk with their doctor about their clinical trial eligibility. LLS's clinical trial navigation service can help patients find and enroll in an appropriate clinical trial. **Patients and caregivers can access this service free of charge by calling (800) 955-4572 or going to www.lls.org/informationspecialists.**

# 3 Volunteer and Campaign with LLS

LLS is the world's largest voluntary health agency dedicated to blood cancer, and volunteers are the heart and soul of the organization. Volunteers help in so many ways – supporting patients, advocating for better access to treatments, making fundraising efforts both fun and effective, and always setting the standard for compassion and kindness.

With 56 chapters across the country, there are many ways to volunteer – from fundraising events to patient services and family support groups to education programs to community outreach. **To learn how to get involved, visit www.lls.org/volunteer.**

LLS has been a pioneer in creating theme-driven campaigns that raise funds for blood cancer research and support while engaging the public with our mission. Our signature campaigns include:

During a sports physical in 1991, when she was 15 years old, **Jennifer Swanton** was diagnosed with leukemia. In 2001, she joined Team In Training and has completed a marathon, triathlon and century ride.

LEUKEMIA & LYMPHOMA SOCIETY® | TEAM IN TRAINING® teaming up to make someday today

**Team In Training** is the original sports training program for charity, providing "teammates" a unique opportunity to experience team camaraderie and experienced coaching, personal discovery and mastery, while supporting groundbreaking discoveries in research. Team In Training raised $32 million in fiscal year 2016, and has raised more than $1.5 billion over the past 29 years, training more than 650,000 teammates. As it approaches its 30th year, Team In Training is once again leading the pack with new offerings to excite the next generation. No longer just marathons, Team In Training's portfolio includes high-caliber events in cycling, hiking and climbing; most recently, Team In Training's first "Climb 2 Cure" teams summited Mount Kilimanjaro.

LEUKEMIA & LYMPHOMA SOCIETY® | LIGHT THE NIGHT®

**Light The Night** is a powerful campaign bringing light to the darkness of cancer. One million friends, families and co-workers gather together, carrying illuminated lanterns in 150 inspirational evening walks, to celebrate, honor or remember those touched by cancer. Light The Night had a record year in 2016, raising $68.5 million for blood cancer research; the campaign has raised more than $625 million since 1999. In 2016, Light The Night introduced Random Acts of Light, engaging celebrities and local heroes to surprise people touched by blood cancers with special meetings, to help brighten their lives during a dark time.

LEUKEMIA & LYMPHOMA SOCIETY® | STUDENT SERIES learning to make someday today

**Student Series** is a service learning, character education and philanthropy program where students gain the unique experience of helping thousands of people in their fight against blood cancers. Programs are tailored to each school level so students can grow with Student Series and have an even greater impact on the lives of patients each year. More than 13 million students and 850,000 educators in 27,000 schools across the U.S. participate annually. Since it began, students have raised more than $315 million to fund breakthrough therapies and patient services, with more than $26 million in 2015-2016 alone.

LEUKEMIA & LYMPHOMA SOCIETY® | LEUKEMIA CUP REGATTA® sailing to make someday today

**Leukemia Cup Regatta** is a thrilling series of sailing events that combines the joy of boating with the important task of raising money to cure cancer. At events held at yacht clubs across North America, skippers register their boats and recruit friends and colleagues to help crew and to raise funds. Crew members seek donations from friends, family, co-workers and employers to sponsor their boat. More than $58 million has been raised through the Leukemia Cup Regatta series for lifesaving research and patient services since its start 30 years ago. The fiscal year 2016 Leukemia Cup Regatta campaign grossed nearly $3.9 million.

LEUKEMIA & LYMPHOMA SOCIETY | MAN & WOMAN OF THE YEAR®
leading the way to make someday today

**Man & Woman of the Year** is a truly unique fundraising campaign. In an annual competition in communities across the country, candidates compete in honor of children who are local blood cancer survivors, the Boy & Girl of the Year, by raising funds for LLS. By engaging influential community leaders, Man & Woman of the Year taps into the spirit of innovation and entrepreneurship that has allowed LLS to achieve great success in funding groundbreaking research to advance cancer cures. The winners receive LLS's "Man & Woman of the Year" titles. Every dollar raised counts as one vote and the titles are awarded to the man and woman with the most votes at the end of 10 weeks. Top local fundraisers become eligible to win national titles. In 2016, candidates raised $38 million.

## 4 Support Blood Cancer Cures

LLS funds research based on the most urgent medical needs, provides education and support to patients, and advocates for policies that ensure affordable, coordinated care. Every dollar invested is used in a number of ways in the fight against blood cancers:

- ✓ Helping patients and their families. LLS provides information and support to guide patients from diagnosis through survivorship, and helps ensure access to current treatments and clinical trials.
- ✓ Improving access to affordable, quality and coordinated care. As the voice for all blood cancer patients, LLS achieves groundbreaking results for patients by advocating for legislation at the state and federal level.
- ✓ Encouraging young scientists to pursue blood cancer research. Grants to young scientists help grow research talent even as federal research funding becomes increasingly limited.
- ✓ Investing in immunotherapies and precision medicine. Developing the most cutting-edge immunotherapy and precision medicine treatments in order to find cures.

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers
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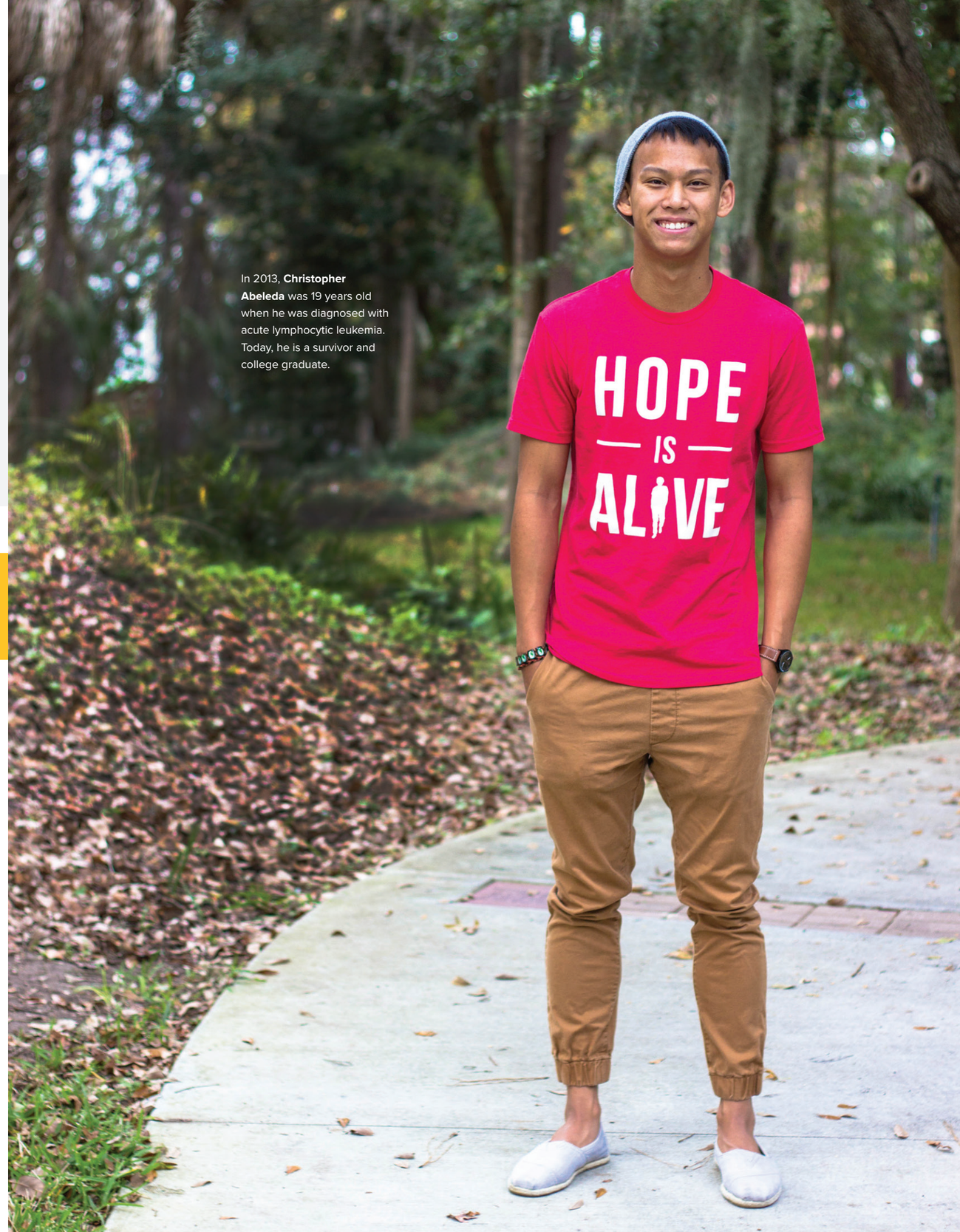
**donate.lls.org**

DESIGN: 3RD EDGE




In 2013, **Christopher Abeleda** was 19 years old when he was diagnosed with acute lymphocytic leukemia. Today, he is a survivor and college graduate.

## OUR MISSION

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

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

someday is today®

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