Changing the Landscape of Pediatric Leukemia Care

A 2024 Impact Report prepared especially for Lea's Foundation

Connecticut Children's FOUNDATION

To Lea's Foundation Board Members:

One of the hardest things we have to do is tell a parent their child has cancer. Last year, we had that conversation more than 120 times at Connecticut Children's Center for Cancer & Blood Disorders. But there was also a ray of light last year. One of the clinical trials in which we were participating closed, and the Food and Drug Administration approved the drug—blinatumomab—for adult and pediatric patients with B cell acute lymphoblastic leukemia.

Your steadfast support over the years allowed us to participate in that clinical trial, and patients here at Connecticut Children's benefited from this lifesaving therapy. You'll meet one of them, Adrianna, in this report.

We never stand still, of course. We continue to bring in early phase trials for leukemia in an effort to better help every child diagnosed with leukemia at Connecticut Children's. Cure rates for patients with certain leukemias have greatly improved, but when a patient relapses, the outcome is often not as positive. To that end, we are expanding our research program this year by joining with Dana-Farber Cancer Institute in a research consortium. This will give us greater influence in how future clinical trials are developed.

Your generosity and ongoing commitment are what allow us to enroll patients in lifesaving clinical trials, advance leukemia research, and offer hope to the children and families who come to the Center for Cancer & Blood Disorders. It takes a team to deliver world-class cancer care, and we are deeply grateful that you have chosen to be part of our team. Thank you.

Sincerely,

Michael Isakoff, MD
Hartford Whaler's Endowed Chair in Children's Cancer
Division Head, Center for Cancer & Blood Disorders
Director, Sarcoma Program
Director, Reid R. Sacco Adolescent and Young Adult Cancer Program
Medical Director, Connecticut Children's Clinical Trials Unit

Meet Adrianna

Adrianna was in kindergarten when she was diagnosed with acute lymphoblastic leukemia (ALL).

Connecticut Children's pediatric hematologist/oncologist Natalie Bezler, MD, offered her parents, Michelle and David, the opportunity to enroll Adrianna in the blinatumomab clinical trial. The clinical trial would give Adrianna access to an experimental therapy that would otherwise be unavailable.

Participating in the trial also meant contributing to a greater understanding of ALL. "Her blood, her fluids, her data would be used along with everybody else in the clinical trial to hopefully find better treatments for kids, to understand the disease better," said Michelle, "and not have future kids have to go through the intense treatment that Adrianna had to go through."

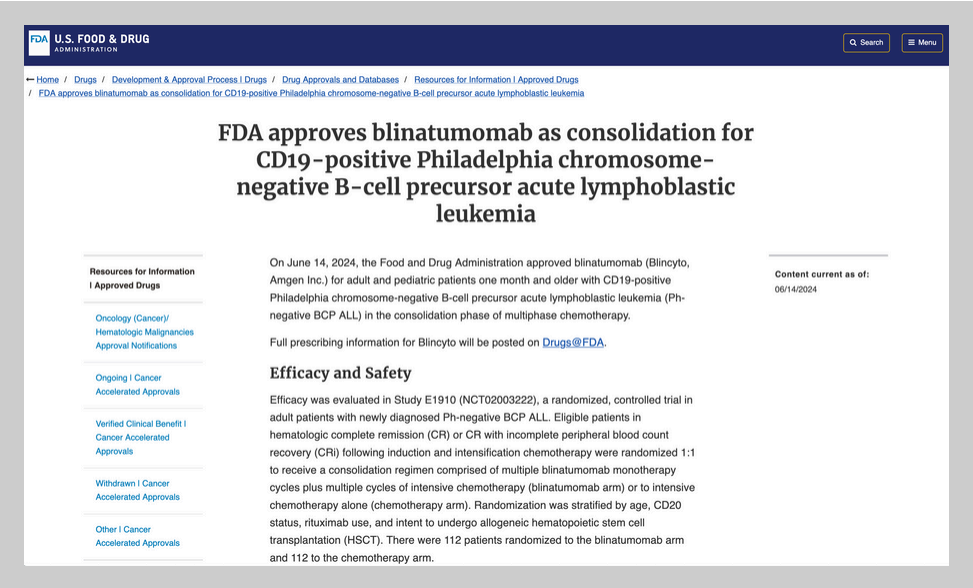
The next day, Adrianna and her mom dyed her hair pink and purple, a sign of the spirit and spunk that would carry her through the months of grueling treatment that were about to begin. During her first 14 days in Connecticut Children's Center for Cancer & Blood Disorders, she underwent blood transfusions, surgery to insert a chemotherapy port, chemotherapy, and lumbar punctures to collect samples of cerebrospinal fluid.

In less than a year, Adrianna spent 96 nights in the hospital. To make her room homier, her parents brought in their own sheets and blankets and decorated the room. “We built forts for her. She did her own yoga. She would make paper slippers,” Michelle said. “But there were definitely days when she did not feel good. There were days that broke my heart.”

In between hospital stays, she had clinic visits for chemotherapy, with some phases requiring Adrianna to come to clinic four days a week for two weeks, then two weeks off. There were complications and hard days along the way. She developed mucositis, inflammation of the lining of the mouth and gut that can lead to painful sores throughout her body. She was on a morphine pump to combat the pain at one point. There were birthday parties missed when her blood counts weren’t high enough to be discharged and times when she couldn’t walk and had to be carried everywhere.

Adrianna is now nine years old and in remission. Her final chemotherapy treatment was in June 2023 and she hasn’t been admitted to the hospital in over a year. She still goes to Connecticut Children’s once a month for follow-up visits. The blinatumomab affected Adrianna’s ability to produce her own antibodies. Until her body begins to create antibodies on its own again, she needs immunoglobulin infusions to strengthen her immune system.

Despite the hardship of cancer treatment, her zest for life still shines. She likes gymnastics, dancing and singing. She’s a Swiftie. “She’s got quite the personality. She’s a special kid,” said David. **“I used to tell her, when she was sitting in the hospital, that 99 percent of people have never gone through what you’re going through. And you’ve beaten it. You can always look back on that as a source of strength.”**



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FDA approves blinatumomab as consolidation for CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia

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On June 14, 2024, the Food and Drug Administration approved blinatumomab (Blincyto, Amgen Inc.) for adult and pediatric patients one month and older with CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia (Ph-negative BCP ALL) in the consolidation phase of multiphase chemotherapy.

Full prescribing information for Blincyto will be posted on Drugs@FDA.

Efficacy and Safety

Efficacy was evaluated in Study E1910 (NCT02003222), a randomized, controlled trial in adult patients with newly diagnosed Ph-negative BCP ALL. Eligible patients in hematologic complete remission (CR) or CR with incomplete peripheral blood count recovery (CRi) following induction and intensification chemotherapy were randomized 1:1 to receive a consolidation regimen comprised of multiple blinatumomab monotherapy cycles plus multiple cycles of intensive chemotherapy (blinatumomab arm) or to intensive chemotherapy alone (chemotherapy arm). Randomization was stratified by age, CD20 status, rituximab use, and intent to undergo allogeneic hematopoietic stem cell transplantation (HSCT). There were 112 patients randomized to the blinatumomab arm and 112 to the chemotherapy arm.

Content current as of: 06/14/2024

Landmark Approval for Blinatumomab

Adrianna was one of 4,381 patients across 226 Cancer Oncology Group (COG) member sites enrolled in the blinatumomab trial. Over the course of the trial, Connecticut Children's enrolled 19 patients and six of those patients were randomized and received blinatumomab. On June 14, 2024, the FDA approved blinatumomab for adult and pediatric patients one month and older with CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia (ALL).

The study found that blinatumomab, when added to chemotherapy, produced significantly superior outcomes, compared to chemotherapy alone. The clinical trial increased survival rates for patients with high-risk disease by nearly 10%. An improvement of that magnitude is almost unheard of in cancer treatment.

Your commitment was critical to the success of the blinatumomab trial.

Thanks to Lea's Foundation, we had the resources necessary to participate in the blinatumomab trial. We were also able to enroll Adrianna and other patients at Connecticut Children's and to contribute data that ultimately led to the FDA approval. The impact of your support of leukemia research extends far beyond the walls of Connecticut Children's. Thanks to you, lives of patients across the country were saved during this trial—and even more children and young adults around the world will win their battles against leukemia in the future.

Dana-Farber Cancer Institute Tumor Bank

Natalie Bezler, MD, has continued her collaboration with Dana-Farber Cancer Institute to develop a tumor bank and conduct genomic profiling for various forms of leukemia. The tumor bank collects and stores bone marrow samples from newly diagnosed patients, as well as patients with relapsed or treatment-resistant acute or chronic leukemia. It also includes samples from patients with myelodysplastic syndrome, myeloproliferative syndrome, and other bone marrow disorders. Last year, Connecticut Children's enrolled 29 patients in the tumor bank.

Dana-Farber Cancer Institute Research Consortium

We are excited to expand our research program at the Center for Cancer & Blood Disorders by joining with Dana-Farber Cancer Institute in a research consortium. There are 13 hospitals participating in this consortium, a size that will give us the power to influence what happens in clinical trials and have a say in how they are developed.

Our work is possible because you care enough to support it.

Clinical trials are the frontline of cancer treatment and where progress in care occurs. Your generosity is changing the landscape of leukemia care, not just for children in Connecticut, but for children and young adults around the world.

You make this happen. Thank you.


THANK
YOU!


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