

New drug for children with high-risk leukemia

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Each year, approximately 4,500 children in America are diagnosed with leukemia, according to the Leukemia and Lymphoma Society. A potentially deadly cancer of the blood, it is the most common cancer in children.

"Modern medicine can cure eight out of 10 cases of childhood leukemia, so parents can still be hopeful when they hear a diagnosis," says Dr. Shai Izraeli of Tel Aviv University's Sackler School of Medicine and Sheba Medical Center. "Our research gives hope and life to the 20% who might not make it as well as those who may experience a relapse."

The first researchers to discover a mutation of the JAK2 protein in patients with Down syndrome, the Tel Aviv University team suspected that this protein might also be linked to other disorders and diseases — and they were right. Based on the successful results of this research a drug that is already in clinical trials for a blood disease common in adults may be relevant for acute childhood leukemia. If initial trials go well, the drug could fast-track through approvals and could be available for treating [children](#) with leukemia in only a few years.

The recent findings are based on Dr. Izraeli's original discovery of the JAK2 in Down syndrome, published recently in the prestigious medical journal *The Lancet*.

Finding a model in children with Down syndrome

According to Dr. Izraeli, a similar mutation of the JAK2 in Down syndrome and leukemia causes Polycythemia Vera, a disease common in adults that leads to the overproduction of blood. This discovery of a similar mutation in a subset of pediatric leukemia cases may provide a path to new life-saving medication options.

Dr. Izraeli first discovered JAK2 mutations in children who initially suffered from Down syndrome and subsequently developed leukemia (a child with Down syndrome is 20 to 30 times more likely to develop leukemia during childhood than a child without it). Dr. Izraeli was then inspired to screen for gene mutations that could result in increased proliferation of cells. In collaboration with the iBFM Study Group, a European [childhood leukemia](#) consortium, 90 cases of [Down syndrome](#) leukemia from all over Europe were studied. A JAK2 mutation was found in 20% of these cases.

The discovery represents a unique biological phenomenon. "This is perhaps the first example of two very similar — but different — [mutations](#) that apparently do the same thing in a cellular protein. But they're associated with two completely different disorders, one that causes polycythemia in adults and the other that causes leukemia in children," says Dr. Izraeli.

"Those children at the highest risk for leukemia may be treated with inhibitors of JAK2," he says. "And because of the existence of polycythemia in adults, there are already drugs to fight polycythemia entering into trials as we speak. We will know in just a few years what these drugs are capable of."

An alternative to chemotherapy

Dr. Izraeli says the discovery offers "potential hope" to children who suffer from leukemia. "JAK2 inhibitors are not based on chemotherapy.

The first experiences with these treatments show very few side effects. All that researchers need to do is to expand these clinical trials to children and adults with high-risk leukemia — and that can happen relatively quickly," says Dr. Izraeli.

Dr. Izraeli explains that typical chemotherapies for leukemia also have a high "toxicity cost." Children with [leukemia](#) are treated with 10 to 12 different chemotherapies over a period of two to three years. Some of them have long-term and irreversible damage, such as neurological, heart, bone problems and sterility. Researchers looking for viable alternatives may turn to Dr. Izraeli's research as a promising avenue for success.

Source: Tel Aviv University ([news](#) : [web](#))

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