FDA Approves Ivosidenib (Tibsovo®), a Targeted Drug, for Acute Myeloid Leukemia

By Julie Grisham, **Thursday, July 26, 2018**

Leukemia Service Chief Martin Tallman (shown here with nurse practitioner Bernadette Cuello) is leading clinical trials for new leukemia drugs.

Summary

> Ivosidenib is the first drug in a class called IDH1 inhibitors to receive FDA approval. It works by targeting a defect in cancer cells.

The US Food and Drug Administration has approved the drug ivosidenib (Tibsovo®) for the treatment of certain people with **acute myeloid leukemia** (AML) that has stopped responding to other therapies. Memorial Sloan Kettering hematologist-oncologist **Eytan Stein** was a co-leader of the study that led to the drug's approval. The results of the trial were **published last month** in the *New England Journal of Medicine* (*NEJM*), and the drug was approved on July 20, 2018.

Ivosidenib is the first drug in a class called IDH1 inhibitors to receive FDA approval. It works in a similar way as enasidenib (Idhifa®), a drug **approved in 2017** to treat AML that's driven by a mutation in a related gene, *IDH2*. Both drugs represent a "new approach to treating cancer," says Dr. Stein.

"Instead of killing cancer cells, like other leukemia drugs, it reprograms them and transforms them into normal, healthy, functioning cells," he says.

About 10% of people with AML have mutations in the *IDH1* gene, and another 15% have *IDH2* mutations. These mutations are also found in other types of leukemia as well as **myelodysplastic syndromes**, **glioblastoma**, and **bile duct cancer**. Targeting these mutations is a growing area of cancer drug development.

MSK President and CEO **Craig Thompson** led the basic science research that explains how *IDH1* mutations drive AML, in collaboration with MSK physician-scientists **Ross Levine** and **Omar Abdel-Wahab**. The Peter and Susan Solomon Family Foundation supported that research, which was first reported in 2010. The investigators found that the mutations produce a cancer-causing enzyme called hydroxyglutarate (2HG). This enzyme stops the development of the blood cells called myeloid cells when they are in an immature form, which leads to leukemia.

Ivosidenib brings down the level of 2HG, so the blood cells can begin to develop normally again.

The *NEJM* study was a multicenter phase I trial that reported data on 125 people whose cancer had stopped responding to other treatments. The researchers found that of those treated with ivosidenib, almost 42% responded. Nearly 22% had a complete remission, meaning that their cancer was no longer detectable. The overall

survival was longer than what would be expected for people with this stage of AML, and severe side effects were rare.

MSK Leukemia Service Chief **Martin Tallman** also participated in the study.

Ivosidenib and enasidenib are both made by Agios Pharmaceuticals.

Comments

> Commenting is disabled for this blog post.

Diane Wilkens

Sep 30, 2018 • 7:12 PM
Would this Drug also work with chronic myeloid leukemia CML?

Memorial Sloan Kettering

Oct 1, 2018 • 6:31 PM
Dear Diane, this drug is not used to treat CML because CML is caused by a different kind of genetic mutation. There are several other targeted therapies that are approved for the treatment of CML. Thank you for your comment.

Irene Bradberry

Nov 19, 2018 • 2:16 AM
I have MDS w low counts in my HGB and ANC. My doc is try to get me approved for Tibsovo. I have the IDH1 mutation. If I use this now will I be able to use Dacogen whenever the Tibsovo stops being effective?

Memorial Sloan Kettering

Nov 19, 2018 • 8:55 AM
Dear Irene, we're sorry to hear about your diagnosis. This is something you should discuss with your doctor. If you are interested in coming to MSK for a consultation, you can call **800-525-2225** or go to **https://www.mskcc.org/experience/become-patient/appointment** for more information on making an appointment. Thank you for your comment and best wishes to you.

William Stanbach

May 12, 2019 • 1:34 PM