Cost barriers limit promising GM blood cancer treatments

Cancer treatments that genetically modify patients' blood cells to target the disease have shown amazing results in clinical trials. Now drug companies and biotechs must overcome big hurdles to get them into hospitals, including their potential cost.

In two separate clinical trials—sponsored by Novartis AG of Switzerland and Seattle-based biotech Juno Therapeutics Inc.—almost 90 percent of patients saw their leukemia disappear after being given experimental so-called CAR T-cell therapies.

There are still big unanswered questions about CAR therapies: one is how long they last.

That is hard to tell because of the small numbers of patients treated so far, and because many of those whose cancer went into remission after the CAR therapies subsequently became eligible for stem-cell transplants—which can themselves prolong survival.

Another concern is a potentially dangerous side effect called "cytokine-release syndrome," an immune response which shows the therapy is working, but which can cause a sharp drop in blood pressure and surge in the heart rate.

The deaths of two patients in a Juno-backed Sloan-Kettering trial in March caused a temporary halt in the study because of worries over these immune responses.

"Patients need to be healthy enough to combat that side effect," says Bishop, who thinks it is now manageable. The trial is recruiting patients again, excluding those with a risk of heart failure, and giving those with very advanced leukemia fewer modified cells.

Read full, original article: New Costly Cancer Treatments Face Hurdles Getting To Patients