DOCTOR: So the correct study it was a phase 3 trial, meaning a large registration trial, investigating a novel agent called regorafenib in patients who had what we call treatment refractory colorectal cancer, in patients who have kind of gone through all the available standard therapies for this disease with good performance status, and then had access to this drug within the clinical trial.

760 patients were recruits within 11 months in this indication, 35 at Mayo Clinic, the largest cohort in the United States. And the trial randomized patients, meaning allocated patients to either getting regorafenib or placebo, meaning a dummy drug, 2 to 1 randomization. Two out of three patients had a chance to get access to this oral potentially active agent.

So number one it is a novel agent. It's something that hasn't been tested before, and it has a unique mechanism action in the sense that it blocks a lot of pathways which tumor cells use to activate themselves to become more aggressive to spread throughout the body. And it's particularly interesting for patients who have gone through several lines of therapy, have gone through several normal conventional treatment options that are available.

Because we know that tumor cells change over time and become sometimes more aggressive, sometimes more sophisticated in circumventing our treatment approaches. So using a drug like regorafenib, which inhibits a lot of these pathways, a so-called multi-kinase inhibitor, makes a lot of sense in this setting and has proven to be effective.

You know the unmet need is I think best exemplified by the observation how fast we recruited this trial. So keep in mind this was a 2 to 1 randomization to a placebo. So people might have questioned you, are patients willing to really embrace this randomization to potentially get a sugar pill, a dummy drug? And we projected the accrual of about 700 patients to last for about 26 months.

Now the trial completed accrual worldwide within 11 months. We were 15 months ahead of schedule, and we enrolled at 760 patients. We kind of overshot, because patients just wanted to get on this trial, which clearly indicates this unmet need. And there is a patient population that is willing to, and desperate of course, to even travel far distances. I have patients that drive in from Kansas City every two or three weeks just to be able to participate in this clinical trial. So there is a clear unmet need, clear orphan indication for this drug.

So the result needs to be put in context with the fact that when patients have run out of treatment options, their overall survival, their prognosis is very poor. In our study, median overall survival for those patients who did not have access to the drug was just five months. So we improved the median overall survival with this drug from 5 to 6.4 months. Now 1.4 months doesn't seem to be a lot, but for a patient who only lives five months, it could be a lot.

Now this only affects the median difference. There are some patients actually benefited quite a lot more. Patients on this trial for about 10 months even where we controlled the progression, the aggressiveness of this cancer with a pill, two or three pills a day for some time, depending on the side-effects we saw. And for some of these patients the benefit was much larger than 1.4 months median.

I think it's better to look at the overall population and not just focus on this one data point, 1.4 months. And when we look at the overall patient population, I think it's fair to say the best measure is that the risk of dying on the study from colorectal cancer was reduced by 23%. And the risk of tumor progression was reduced by 50%. So that is, in my eyes, as a clinical oncologist, a meaningful difference which will hopefully allow us to eventually get this drug approved by our FDA.

This drug regorafenib is being utilized now in clinical trials, in earlier lines of therapies, not in the kind of refractory patient population, but in combination with chemotherapy, standard treatment in earlier treatment lines of therapy. And it's also being investigated in other tumor types.

So there's a large trial ongoing right now targeting patients with so-called gist tumors, gastrointestinal stromal tumors, which already have treatment options. But again, here is a fallback position. Regorafenib is being used. We'll hopefully see the results of this trial actually as early as this year.

As an oncologist who sees patients every day who are desperate and running out of treatment options, are still in excellent performance status, they come to me and ask, so what's next? What else do you have? I've gone through of these treatment options. They come from across the country to ask, is there anything else you can offer? And until recently, we didn't have anything realistic to offer outside of experimental therapies, et cetera.

Now this drug is the first drug with a very innovative, novel treatment approach and mechanism of action that has shown to improve the survival of patients in this last-line setting in a meaningful way for eight years. So this is as a clinical oncologist, a quite exciting time that we kind of move the bar a little bit higher and hopefully can improve survival for individual patients a bit longer, as a next stepping stone for future improvements in our therapy.