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Released: 06/20/2023

Valid until: 06/20/2024

Time needed to complete: 1h 08m

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### Updates in the Field of Non-Advanced Systemic Mastocytosis

#### Announcer:

Welcome to CME on ReachMD. This episode is part of our MinuteCE curriculum.

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#### Dr. Akin:

Hi. I'm Dr. Cem Akin. I'm a Clinical Professor of Medicine at the University of Michigan. And I would like to provide you with a quick update on the approval of avapritinib by Food and Drug Administration for the indication of indolent systemic mastocytosis. This approval occurred on May 22<sup>nd</sup>, and it was based on the results from the PIONEER trial, in which the patients were randomized to receive either avapritinib or placebo. The trial showed that the patients demonstrated significant improvements in their total symptom scores as well as markers of mast cell burden like serum tryptase, bone marrow mass infiltration, and the D816V allele burden. Most patients experienced improvement in their skin lesions. And the drug was overall well tolerated with the most significantly increased side effects being peripheral or eyelid edema, and elevations in alkaline phosphatase.

So what does this mean for the patients? It means that there is a cytoreductive treatment option available there is well tolerated and it leads to symptom improvement if they are already using multiple best treatment or medication options and still remain symptomatic.

Second, a couple of important exclusions are patients with low platelet counts less than 50,000 should not be on this drug due to the observed intracranial bleeds in the advanced trial. And finally, if the patient is planning to start a family, have children, this drug may not be the best for those patients either.

If you would like to hear more about the treatment of non-advanced mastocytosis, please review the episodes in this series. Thank you for your attention.

#### Announcer:

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