Transcript Details

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Next-Generation Horizons in PNH Therapies

Announcer:
Welcome to *Project Oncology* on ReachMD. On this episode we'll discuss how we can achieve better outcomes when managing patients with paroxysmal nocturnal hemoglobinuria, otherwise referred to as PNH, with Dr. Vinod Pullarkat. Dr. Pullarkat is a professor of Hematology and Hematopoietic Cell Transplantation at City of Hope Medical Center in California. Here he is now.

Dr. Pullarkat:
For patient-centric care in PNH, the latest approaches are developments in complement inhibition. So initial drugs that were approved for PNH targeted the latter part of the alternative complement pathway, particularly the C5 inhibitors that were approved first. But over the last couple of years, there are a lot of clinical trials testing novel agents, particularly agents that act more higher up on the alternative complement pathway. And these drugs will be expected to provide better control of hemolysis and improve symptoms of the patients.

As we know, PNH is a disease, which not just causes anemia but also other symptoms and multiple systems related to the hemolysis and the release of free hemoglobin. So with these newer drugs, one will expect that we will have better production of the hemolysis, higher hemoglobin levels, not only that, there will be less complications associated with intravascular hemolysis and the other consequences of that. So one would expect patients to have benefits with regard to the various symptomatologies that accompany PNH.

The testing for PNH has evolved over the last decade or so. There is better flow cytometry techniques, so we can monitor small clones and monitor the size of the clones over a period of time using these techniques. There are techniques in development to determine the complement of activation and help to monitor that, although those are not going to be available at this time.

The other advances are in the area of genomic testing to determine mutations that are associated with PNH and this will help us to separate classic PNH from other situations where PNH clones exist, typically patients with MDS or sometimes patients who are recovering from aplastic anemia. So with the newer technologies, we have a clearer definition of these patient's diagnosis as well as improvement in the ability to monitor these patients, especially during treatment.

So if you ask about the unmet medical needs for patients with PNH, the biggest problem we have with currently approved drugs is incomplete production of hemolysis. So many patients are left with residual anemia as well as some of the other systemic symptoms. Because although the hemoglobins are improved, they continue to have a degree of underlying hemolysis that leads to many symptoms. And often these patients also get transfused periodically for various reasons.

With the newer drugs, particularly the earlier inhibitors of the complement pathway, the factor B inhibitors, factor D inhibitors, these drugs in our experience, from early clinical trials, they provide a more complete production of hemolysis, much more than the C5 inhibitors. So I would expect that the patients will be better off symptomatically, and also just because there is more complete production of hemolysis; there is less long-term complications as well as better quality of life. I think these newer drugs in development will make a huge impact for both for the outcomes as well as for the quality of life for PNH patients.

Announcer:
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