Rapid Responses to Avapritinib (BLU-285) in Mastocytosis

In a phase I trial, patients with advanced systemic mastocytosis, which includes mast cell leukemia, experienced rapid and durable responses, with manageable side effects, following treatment with avapritinib (BLU-285; Blueprint Medicines). Results of the trial to date were presented at the 2017 American Society of Hematology Annual Meeting in Atlanta, GA, held December 9–12.

Until recently, the standard of care for patients with advanced systemic mastocytosis has been the chemotherapeutic cladribine. In April, the FDA approved midostaurin (Rydapt; Novartis) for the treatment of the disease, and “it is catching on as the only FDA-approved option,” said Neil Shah, MD, of the University of California, San Francisco.

In comparison to midostaurin acts rapidly and lacks a high response rate, with just 17% of patients experiencing a complete or partial response.

Clearly, “there is an unmet medical need” for a more effective drug, commented Neil Shah, MD, of the University of California, San Francisco. In comparison to midostaurin, avapritinib is a highly potent and specific oral inhibitor of mutant KIT that harbors activation loop mutants, which play a key role in GIST. Avapritinib is also under study for other diseases can be and probably should be—and we’re all recommending—tested with this agent, but it’s probably limited to just a half a dozen or so KIT-driven diseases,” said DeAngelo. –Suzanne Rose

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Prostate cancer researchers found significant disparities when they submitted identical patient samples to two different commercial liquid biopsy providers, raising the possibility that patients could be prescribed different treatments depending upon which company performs the liquid biopsy (JAMA Oncol 2017 Dec 14 [Epub ahead of print]). The researchers compared Guardant360 (Guardant Health), which sequenced at least part of the coding sequences of 73 genes, and PlasmaSELECT (Personal Genome Diagnostics), which analyzed coding sequences of 64 genes. Just 25 of the 40 patients in the study had at least one genetic mutation reported within the genetic sequences covered by both companies.

Cancer Research UK announced a 5-year drug-discovery collaboration between its subsidiary, Cancer Research Technology (CRT), and Celgene to discover, develop, and commercialize new anticancer treatments. The collaboration is centered on mRNA translation.

The FDA updated the label for nilotinib (Tasigna; Novartis) to include information on discontinuing the drug in patients with chronic-phase Philadelphia chromosome–positive chronic myeloid leukemia who have achieved a sustained molecular response (MR4.5) to it after at least 3 years of treatment. Criteria for monitoring patients who discontinue nilotinib are also spelled out.

The Institute for Clinical and Economic Review released its Draft Evidence Report comparing the effectiveness and value of two chimeric antigen receptor T-cell therapies—tisagenlecleucel (Kymriah; Novartis) and axicabtagene ciloleucel (Yescarta; Kite/Gilead)—for certain B-cell cancers (available at https://icer-review.org). The independent nonprofit research organization’s report concludes that the therapies “provide gains in quality-adjusted and overall survival over alternative chemotherapies.”