FDA Expedites Potential New Therapy for Rare Lymphoma

(Feb 21, 2013) Patients with a rare and incurable cancer called Waldenstrom’s macroglobulinemia (WM) have a new reason for hope, thanks to the U.S. Food and Drug Administration’s (FDA) decision to grant Breakthrough Therapy Designation for the investigational oral agent ibrutinib, developed as treatment for WM and for relapsed or refractory mantle cell lymphoma (MCL).

“This is a historic day for the WM community,” stated Carl Harrington, President of the volunteer-led International Waldenstrom’s Macroglobulinemia Foundation (www.iwmf.com) which is dedicated to WM disease research and patient support. “We are excited by the FDA’s decision and by the joint commitment of Janssen Research and Development and Pharmacyclics to develop a treatment for our very rare disease that affects 3 per 1 million persons annually in the U.S.” Tom Myers, IWMF Vice President for Research, added “We are proud of the WM patients who are currently participating in clinical trials of ibrutinib.” Both Harrington and Myers are also patients.

This is an important advance in the development of therapy for WM, a rare and incurable type of B-cell lymphoma (http://www.iwmf.com/about-wm/index.aspx) for which no standard of care or FDA-approved treatment exists. It comes on the heels of another important development in WM – the discovery of a genetic mutation (known as MYD88 L265P) that is prevalent in almost all WM patients. The mutation was detected in research performed by Dr. Steven Treon and his group at the Dana-Farber Cancer Institute in Boston, MA, and funded in part by the IWMF. Dr. Treon’s group has also discovered that the mutation impacts some of the same cellular pathways targeted by ibrutinib. “Without the impetus of advocacy for research, seed funding by the IWMF, and the dedication of top-notch researchers around the world working on our rare disease, this would not have been possible,” said Harrington.

Breakthrough Therapy Designation is intended to expedite the development and review time for potential new medicines that can treat a serious or life-threatening disease and that demonstrate substantial improvement over existing therapies. It was signed into law on July 9, 2012, as part of the U.S. Food and Drug Administration Safety and Innovation Act (FDASIA). Since then, three drugs have achieved this status, with ibrutinib being the first approved for cancer treatment.

About the International Waldenstrom’s Macroglobulinemia Foundation (IWMF)
The International Waldenstrom’s Macroglobulinemia Foundation (IWMF) had its roots in a patient support initiative begun by a retired pharmacist in 1994 who had WM, and was incorporated in Florida as a 501(c) (3) tax exempt non-profit organization under its current name in 1998 for the express purpose of offering educational information and caring support to Waldenstrom’s macroglobulinemia (WM) patients. Since the year 2000 the Foundation has expanded its scope to include the funding of research that will lead to better treatment therapies and eventually a cure.

The Foundation is unique among cancer support and research organizations in that it is entirely operated by a volunteer Board of Trustees, most of whom are WM patients. The Board Advisor is Dr. Robert A. Kyle of the Mayo Clinic, who also serves as a Trustee and is a noted international expert in WM and multiple myeloma.

For more information regarding WM, the IWMF, and the impact of ibrutinib on WM patients, contact Carl Harrington, President of the IWMF, at carlh@comcast.net.

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