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June 20, 2014

Barbara Wedehase, MSW, CGC
Executive Director, National MPS Society
PO Box 14686
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Dear Barbara,

We would like to provide you with an update on the Shire Sanfilippo B program, in response to questions we have received.

In April 2012 Shire enrolled the first participant into a multi-center observational, prospective natural history study of Sanfilippo Syndrome Type B (MPS IIIB) (HGT-SNB-088). The last patient enrolled in the study in September 2013, making a total of 19 patients. The period of observation in this study is 12 months for each patient.

The study was designed to evaluate the natural course of disease progression in individuals with MPS IIIB who are untreated, and to identify potential endpoints that may be utilized in future treatment trials of MPS IIIB using predefined assessments including standardized clinical, biochemical, neurocognitive, developmental, and imaging measures.

Upon study completion, which is estimated to occur around the end of 2014, the data will be analyzed and reported. A full publication is anticipated to occur as soon as possible after completion of the study, so that investigators and scientists engaged in developing treatments for MPS IIIB will be able to use this information.

The commitment of families to participate in such a study, with no guarantee of a successful therapeutic development, is deeply appreciated. Shire had a therapeutic development program for MPS IIIB at the beginning of this natural history study. However, as a result of difficulties encountered in the development of the program, we are no longer pursuing the development program in MPS IIIB.

We remain committed to the MPS Society and patients with MPS IIIB in completing the natural history study and sharing the results from this study in the hope that this will provide a foundation to explore avenues for therapeutic intervention.

Yours sincerely,

Philip J. Vickers, Ph.D.
Global Head of Research and Development
Shire