Shire HGT Metachromatic Leukodystrophy (MLD) Program Update
19 February 2010

Shire HGT had planned to initiate a Phase 2/3 study with intravenous (IV) enzyme replacement therapy (ERT) for treatment of MLD in 2009. However, clinical data from the ongoing Phase 1/2 extension study, collected on an ongoing basis over the last few years, demonstrates that the IV route of administration is not succeeding. Therefore, Shire has decided to suspend further development of an IV formulation of arylsulfatase A (ASA) derived from CHO cells, also known as HGT-1111. Shire plans to share these clinical data publicly at an upcoming scientific meeting and is working closely with the clinical investigators caring for participants continuing to receive HGT-1111 to evaluate next steps for these patients.

Going forward Shire believes that direct delivery to the central nervous system (CNS) offers the best chance for development of a successful treatment for MLD. Shire plans to develop HGT-1110, a formulation of ASA derived from human cells and compatible with direct CNS delivery for MLD patients. This human cell line has been used successfully by Shire for the development of other ERTs for Hunter syndrome, Fabry disease, and type 1 Gaucher disease. Shire's CNS platform was recently advanced with the initiation of a Phase 1/2 trial using direct delivery of idursulfase to the CNS in Hunter patients. Development of the HGT-1110 formulation suitable for direct delivery to the CNS is ongoing, and preclinical studies are planned for 2010.

Shire is committed to the MLD community. We continue to work diligently to bring a much-needed therapy to patients and their families.

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http://MLDfoundation.org/pdfs/Shire-HGT_HGT-1111_statement_2010-02-19.pdf