spotlight on health

Orphan Drugs Give Patients With Rare Illnesses New Hope

(NAPSA)—If you or someone you care about is ever one of the estimated 25 million Americans with a rare disease, there could be good news. Legislation known as the Orphan Drug Act (ODA), now celebrating its 25th anniversary, has successfully spurred innovation and encouraged companies to produce the medicines needed to treat these rare conditions.

Consider the case of Judy Phelps. Now 50, she once doubted that she would ever have the typical, busy but happy life that she leads today with her husband and four young children.

Throughout most of her adult life, Phelps endured countless hospital visits, underwent 13 abdominal surgeries and remained paralyzed for six weeks before physicians finally diagnosed her condition as acute porphyria, solving a nearly two-decade-old medical mystery.

The acute porphyrias are a group of genetic disorders associated with enzyme deficiencies affecting heme production—the component of hemoglobin that carries oxygen throughout the body. Depletion of the heme pool caused by certain triggers in individuals with an enzyme deficiency results in an overproduction of porphyrins and their precursors. A buildup of these chemicals in vital organs can cause acute, sometimes life-threatening attacks.

This group of diseases is frequently overlooked or misdiagnosed because porphyria can present with wide-ranging symptoms that mimic other conditions. Symptoms may include abdominal pain, muscle weakness, vomiting, seizures and potential mental impairment. Left untreated, it can result in permanent neurological damage.

Some historians have speculated that members of the British royal family suffered from this hereditary disease, including Mary Queen of Scots, who passed it on to her son, King James I of England. George III had a particularly severe form of porphyria, which is thought to have affected his ability to make sound judgments, which in turn could have helped trigger the American Revolution. Other affected royals included Queen Anne of Great Britain; George IV of Great Britain—son of George III; and George IV's daughter, Princess Charlotte.

There is hope today for patients thanks to a simple urine test and Panhematin® (hemin for injection), the first drug approved under the ODA as a treatment for the acute porphyrias. Thanks to a proper diagnosis and treatment, Phelps says her porphyric attacks are under control and she is leading a normal life.

For more information about porphyrias, visit www.porphyriafoundation.com.

Note to Editors: Panhematin® (hemin for injection) should only be used by physicians experienced in the management of porphyrias in hospitals where the recommended clinical and laboratory diagnostic and monitoring techniques are available. Panhematin therapy should be considered after an appropriate period of alternate therapy (i.e., 400 g glucose/day for 1 to 2 days). Panhematin (hemin for injection) is indicated for the amelioration of recurrent attacks of acute intermittent porphyria temporally related to the menstrual cycle in susceptible women. Manifestations such as pain, hypertension, tachycardia, abnormal mental status and mild to progressive neurologic signs may be controlled in selected patients with porphyria. Panhematin is not indicated in porphyria cutanea tarda. Panhematin is made from human blood and therefore may contain infectious agents, such as viruses, that can cause disease, including the Creutzfeldt-Jakob disease. Drugs such as estrogens, barbituric acid derivatives, steroid metabolites, and anticoagulants should be avoided during Panhematin therapy. Because phlebitis has been reported after administration of Panhematin through small arm veins, a large arm vein or a central venous catheter should be utilized for administration. Please see full prescribing information and boxed warning at www.ovationpharma.com.