COUNCIL FOR BILE ACID DEFICIENCY DISEASES JOINS NORD AND AHRQ

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February 12, 2013

TO: BILE ACID DEFICIENCY DISEASES: PATIENTS AND FAMILIES

On January 25th, the Council For Bile Acid Deficiency Diseases (The Council) sponsored the participation of a North Carolina parent caregiver of a patient with bile acid deficiency disease in a National Organization for Rare Disorders (NORD) and the U.S. Government Agency for Healthcare Research and Quality (AHRQ) focus group. The focus group was limited to only nine (9) parents/caregivers identified by various rare disease advocacy groups; therefore, this was a meaningful opportunity for the Council to sponsor advocacy and have a meaningful impact.

The Focus Group, led by Dr. Aaron S. Kesselheim, M.D., Brigham and Women's Hospital, Boston, MA, asked these nine advocates/caregivers to discuss their experiences with available therapies, processes to develop new drugs, patient registries and post-marketing studies. The intent of the Focus Group discussions with parents/caregivers was to assess the risks and rewards associated with research in rare diseases. The opportunity for the North Carolina parent/caregiver of a patient with bile acid deficiency disease focused attention on the need for development of new therapies for this extremely small number of patients. The parent/caregiver also discussed the support from the Council for Bile Acid Deficiency Diseases she had received and will continue to mobilize parents/caregivers as advocates.

The Council is a strong advocate for the development of new therapies and has written the U.S. Food and Drug Administration (FDA), Health Canada and the European Medicines Agency (EMA) to advocate for their support and focus their attention on Bile Acid Deficiency Diseases. The Council will seek opportunities to directly interaction and advocate with all national regulatory agencies on behalf of its patients, parents and caregivers. In addition, the Council is advocating for pharmaceutical manufacturers to focus their resources on new therapies and expediting the marketing of therapies.

The Council is a global advocacy group formed in 2012 composed of patients, parents/caregivers, medical professionals and pharmaceutical companies focusing on the needs of patients with Bile Acid Deficiency Diseases. Patients with these Diseases have genetic

mutations of one or more enzymes in their bile acid synthesis and fail to make normal cholic acid molecules. The accumulation of abnormal cholic acid molecules in the blood causes a number of disease pathologies, the most devastating being liver failure and death usually by the age of 4-5 years, if untreated. The Council seeks to provide educational materials on these Diseases and welcomes enquires from the public, parents/caregivers of patients, medical professionals and researchers...info@bileacid.org .

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