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UAB HAS ENHANCED ROLE AS NIH EXPANDS RARE DISEASES CLINICAL RESEARCH NETWORK

BIRMINGHAM, Ala. – The [National Institutes of Health \(NIH\)](#) today announced the second phase of the Rare Diseases Clinical Research Network (RDCRN) including funds for 19 research institutions, among them the [University of Alabama at Birmingham \(UAB\)](#). The network and a data-management coordinating center will be awarded in excess of \$117 million during the next five years. The research conducted with the new funding will explore the natural history, epidemiology, diagnosis and treatment of more than 95 rare diseases.

UAB's focus in the network is to study Rett syndrome, Angelman syndrome and Prader-Willi syndrome. [Alan Percy, M.D.](#), medical director of the UAB [Civitan International Research Center](#), is the lead investigator. Percy, PI of the UAB IDDRC, will also be the chair of the RDCRN steering committee for the next two years.

“The progress made by researchers through the network during the past six years is important and impressive,” said NIH Director Francis S. Collins, M.D., Ph.D. “We have shown that this approach can be a catalyst for progress in meeting the challenge of rare diseases, and we are eager to launch this next phase of the program.”

A rare disease is defined as a disease or condition affecting fewer than 200,000 persons in the United States. Approximately 6,500 such disorders have been identified, affecting an estimated 25 million Americans.

Initially created in 2003, the RDCRN is unique in its approach to addressing rare diseases as a group. Previously, the NIH institutes and centers funded research on individual rare diseases in their respective disease-type or organ domains. The RDCRN is the first program that aims to create a specialized infrastructure to support rare diseases research.

Since its creation, the RDCRN has enrolled more than 5,000 patients in 37 clinical studies in rare diseases. Patient recruitment for clinical studies is a fundamental challenge in rare diseases research because there are typically so few affected patients in any one area. The RDCRN addresses this problem by fostering collaboration among scientists and shared access to geographically distributed research resources. Network consortia have also established training programs for clinical investigators who are interested in rare diseases research.

The direct involvement of patient-advocacy groups in network operations, activities and strategy is a major feature of the RDCRN. Each consortium in the network includes relevant patient-advocacy groups in the consortium membership and activities.

Funds and scientific oversight for the RDCRN will be provided by the NIH Office of Rare Diseases Research and seven NIH Institutes, which also will contribute considerable administrative support to the network: the National Institute of Neurological Disorders and Stroke (NINDS), the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), the National Institute of Allergy and Infectious Diseases (NIAID), the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), the National Institute of Dental and Craniofacial Research (NIDCR), the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) and the National Heart, Lung and Blood Institute (NHLBI). Several institutions also will receive financial support from their associated patient advocacy groups.

About UAB

Known for its innovative and interdisciplinary approach to education at both the graduate and undergraduate levels, the University of Alabama at Birmingham (UAB) is an internationally renowned research university and academic medical center whose professional schools and specialty patient care programs are consistently ranked as among the nation's top 50; find more information at www.uab.edu and www.uabmedicine.org.

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