Rashmi Gopal-Srivastava, J Genet Syndr Gene Ther 2016, 7:5 (Suppl)
http://dx.doi.org/10.4172/2157-7412.C1.008

Rashmi Gopal-Srivastava

National Institutes of Health, USA

The rare diseases clinical research network (RDCRN) program (a model for collaborative research) and resources at NCATS, NIH

Any disease which has a U.S. prevalence of less than 200000 is defined as a rare disease. There are several challenges and opportunities for doing research and drug development for rare diseases. To facilitate clinical research and development of treatments for rare and neglected diseases, the National Center for Advancing Translational Sciences (NCATS) has established several programs. This presentation will focus on the Rare Diseases Clinical Research Network (RDCRN) program for multi-site clinical research involving patient advocacy groups (PAGs) as research partners and will provide a brief overview of some other scientific programs that support therapeutic development for rare diseases. To facilitate natural history studies and clinical trials for rare diseases, the Office of Rare Diseases Research (ORDR) within NCATS has established the RDCRN program. The RDCRN is a successful, collaborative and innovative international clinical studies network of 22 distinct multi-site clinical research consortia and a central Data Management and Coordinating Center (DMCC). The research conducted in this network has involved 144 PAGs and explores the natural history, epidemiology, diagnosis and treatment of more than 200 rare diseases at 267 clinical sites in USA and 17 other countries. Each consortium is required to conduct two multi-site clinical studies on a minimum of 3 related rare diseases, develop a training program for new investigators involve patient advocacy groups as research partners and provide information about rare diseases to researchers, health care providers, patients and general public. The DMCC supports the consortia by supplying infrastructure; user-friendly resources for the public and Web-based recruitment and referral tools; logistical and administrative assistance and data coordination, management and sharing. The goal of RDCRN is to contribute to the research and treatment of rare diseases by working together to identify biomarkers for disease risk, disease severity and activity and clinical outcome, while encouraging the development of new approaches to diagnosis, prevention and treatment. Some other translational programs of NCATS include Therapeutics for Rare and Neglected Diseases (TRND), the Bridging Interventional Gaps (BrIDGs), NIH Chemical Genomics Center (NCGC), Tissue Chip or Microphysiological Systems (MPS) program and New Therapeutic Use program (NTU).

Biography

Rashmi Gopal-Srivastava currently serves as the Director of the Extramural Research Program in the Office of Rare Diseases Research. She leads the Rare Diseases Clinical Research Network (RDCRN), a multidisciplinary national program. She collaborates with 10 NIH Institutes to manage 22 consortia and a central Data Management Coordinating Center. She has been the recipient of the NIH Individual Merit Award numerous times. She also has received recognition from the U.S. Department of Health and Human Services for outstanding community services and NIH's Asian Pacific Islander American Organization's outstanding achievement award for excellence in science. She is an advocate for women in science and has recently been interviewed for the "Women of Color Scientists at NIH" and featured and showcased in "Women in Science at the National Institutes of Health 2007-2008".

Rashmi.Gopal-Srivastava@nih.gov