Orphan Drug Development Guidebook

Building Block U227

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	The NIH Rare Disease Clinical Research Network (RDCRN)
References	https://ncats.nih.gov/rdcrn
Description	The Office of Rare Diseases Research (ORDR) within the National Center for Advancing Translational Sciences (NCATS) along with National Cancer Institute (NCI), National Heart, Lung, and Blood Institute (NHLBI), National Institute of Allergy and Infectious Diseases (NIAID), National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), National Institute of Dental and Craniofacial Research (NIDCR), National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), and the National Institute of Neurological Disorders and Stroke (NINDS). The RDCRN is made up of multiple individual, Rare Disease Clinical Research Consortium (RDCRC) and a Data Management and Coordinating Center (DMCC). The RDCRCs are intended to advance the diagnosis, management, and treatment of rare diseases with a focus on clinical trial readiness. Each RDCRC will promotes highly collaborative, multi-site, patient-centric, translational and clinical research with the intent of addressing unmet clinical trial readiness needs. To date this program has successfully supported 31 individual consortia that conducted research on 238 individual disorders, leading to a greater understanding of rare diseases. Each consortium must study at least three different rare disease and must have patient advocacy groups or patients must be engaged in a significant manner within the consortia.

	Each consortium aims conducting longitudinal/natural history studies and has a focus on clinical trial readiness (e.g., biomarkers, outcome measures).
Category	Developmental Resources Building Block
Geographical scope	United States of America and International
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Informational, potential research collaborators, potential clinical research studies for patients
Stakeholders	• Researchers, • Clinicians, • Patient Advocacy Groups, • Patients, families, caretakers
Enablers/ Requirements	Web access
Output	Specific research studies
Best time to apply and time window	The RDCRN as a network is recompeted once every five years (last competition 2019). Applicants must be within the United States however; international collaborators are allowed. The consortia are open to collaborations with other rare disease researchers and communication with the network or individual consortia are encouraged.
Expert tips	PROs: – Excellent assembled resource CONs: – None