Orphan Drug Development Guidebook

Building Block U228

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	Discovering New Therapeutics Uses for Existing Molecules (NTU) program, National Center for Advancing Translational Sciences (NCATS)
References	https://ncats.nih.gov/ntu
Description	The NTU program aims to improve the process of developing new treatments for diseases by finding new uses for existing therapies that already have cleared several key steps along the development path. Existing or partially developed therapeutic candidates can be repurposed for use in new disease indications. The program aims to bring together assets from pharmaceutical companies (pharma) and new ideas from academic researchers that could produce new treatments more quickly than starting from scratch. NTU can be used for a potential new use of an existing investigational therapy for a rare or common disease. NTU seeks to repurpose existing therapeutic candidates toward disease targets for which they may hold promise, thereby potentially reducing time to development of a therapeutic candidate regardless of indication.
Category	Developmental Resources Building Block
Geographical scope	United States of America

Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	The goal of this building block is to advance the evaluation and development of drug products that are partially developed or developed for a different indication. The purpose is to produce new treatments more quickly than starting from a novel compound. The NTU program brings together assets from participating Pharma companies and the academic biomedical research community. Pharma partnerships (per the website: https://ncats.nih.gov/ntu/about) include: AstraZeneca, MedImmune, AbbVie, Bristol-Myers Squibb, Eli Lilly & Co, GlaxoSmithKline, Janssen Pharma, Meroe BioPharma, Pfizer and Sanofi. Participating Pharma companies must meet inclusion criteria per the published notice: https://grants.nih.gov/grants/guide/notice-files/NOT-TR-14-001.html Investigators may propose testing new therapeutics uses for experimental drugs or biologics across a broad range of human diseases in adult and pediatric populations. Strong applications will include scientific evidence that modulation of a drug for repurposing will have a positive impact on the disease/condition. Investigators with a proposal consisting of strong scientific evidence that modulation of a drug for repurposing will have a positive impact on the disease/condition may consider applying. Please consult published FOAs for additional information regarding scope of the specific awards: https://ncats.nih.gov/ntu/funding
Stakeholders	• NIH NCATS • Participating Pharma companies • Academic biomedical researchers
Enablers / Requirements	Current open funding opportunity announcements (FOAs) are available here: https://ncats.nih.gov/ntu/funding. FOAs vary in scope and target applicants, and potential applicants are urged to read the open announcements carefully.
Output	The NTU program assists with supporting Cooperative Agreements (e.g., U01s, U34s) to enable NTU of candidate therapeutics. Cooperative agreements support testing of the candidate therapeutics at various clinical and pre-clinical phases of study.
Best time to	FOAs are open for varying lengths of time – please consult the website for specifics:

apply and time window	https://ncats.nih.gov/ntu/funding
Expert tips	Please consult the NCATS NIH website for additional information, templates, FOAs, available Pharma assets and application process: https://ncats.nih.gov/ntu/about NCATS staff may also be consulted for additional assistance. PROs: – Available funding for testing of candidate therapeutics for repurposing in a broad range of diseases/conditions. CONs: – Limited funding/opportunities available through peer-review process. Time lag and submission requirements for available FOAs are resource intensive and funding is not guaranteed.