

CONCEPT CLEARANCE RECORD
FY 2019 RESEARCH INITIATIVE — NCATS
January 2018 Concept

CONCEPT TITLE: Clinical Trial Readiness for Rare Diseases

CONCEPT TYPE: New

ASSIGNED DISCUSSANTS: Bartek, Spielberg

OBJECTIVE(S): To support clinical studies that address obstacles to the design of trials needed for rare diseases. Such trials are critical in the development and evaluation of new treatments for rare diseases.

DESCRIPTION: There are over 7,000 rare diseases; most are severe and affect children, and less than 5 percent have an effective treatment. NIH investment into discovery research has contributed to unprecedented opportunities to translate scientific advances into better treatments. Gene therapy and related approaches are examples of opportunities that have resulted from technological advances. However, to evaluate such potentially transformative treatments, researchers, biopharmaceutical companies and regulators need high-quality, recent natural history data, as well as biological and clinical outcome measures fit for the intended purpose. Because such datasets and tools are not available for many rare diseases, these requirements often represent a bottleneck in therapy development, resulting in delays and failure to attract private-sector interest.

The proposed initiative aims to support studies that address these bottlenecks by focusing on specific gaps in natural history data and outcome measures. Given the large number of rare diseases and the great unmet need, in the face of limited available funding, this initiative initially will focus only on a subset of studies in indications for which there are credible clinical development candidates and for diseases for which there are unmet medical needs. The proposed initiative will promote partnerships among academic investigators, industry and patient groups. Additionally, it will encourage interactions with the U.S. Food and Drug Administration (FDA), such as through the Critical Path Initiative. This initiative will encourage the use of existing data standards, tools, information technology platforms, clinical outcomes measures and biomarkers, where available, rather than supporting the discovery or *de novo* development of such tools and resources.

IMPORTANCE: Addressing bottlenecks in rare diseases clinical development will accelerate progress from discovery to patient benefit and thus is directly aligned with the mission of NCATS and NIH.

HISTORY: NCATS and other NIH Institutes and Centers (ICs) are supporting the Rare Diseases Clinical Research Network (RDCRN), which provides larger-scale natural history data for a limited number of rare diseases. The proposed initiative is complementary, as it allows for projects of smaller scope as well as for those not covered under the RDCRN program. The Trial Innovation Network supports excellence and innovation in the conduct of multisite clinical trials. The proposed initiative will help prepare research groups to take advantage of the Trial Innovation Network resources.

Funding opportunity announcements issued by other NIH ICs include [PAR-16-020](#): Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases (U01), re-issued as [PAR-18-534](#): Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases (U01 Clinical Trial Optional), by the National Institute of Neurological Disorders and Stroke; [PAR-18-090](#): Natural History of Disorders

Identifiable by Screening of Newborns (R01 Clinical Trial Optional), issued by the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development and the National Institute of Diabetes and Digestive and Kidney Diseases; and [RFA-FD-16-043](#): *Natural History Studies for Rare Disease Product Development: Orphan Products Research Project Grant (R01)*, issued by FDA. These existing funding opportunities are synergistic, and NCATS is collaborating with the FDA Office of Orphan Products Development and with the relevant NIH ICs on the proposed initiative.

CONCEPT CLEARANCE DATE:

January 11, 2018

COUNCIL RECOMMENDATION:

Council approved as presented.

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