NCATS Toolkit for Patient-Focused Therapy Development

The NCATS Toolkit for Patient-Focused Therapy Development (Toolkit) was developed through a collaboration between NCATS and rare diseases patient groups. The patient groups had expressed an interest in exchanging knowledge and sharing best practices in therapy development. The Toolkit provides a collection of online resources that can help patient groups advance through the process of therapy development and provide the tools they need to advance medical research. As a living, online resource containing tools for and by patient groups, as well as other reliable sources of information, the Toolkit includes information such as how to:

- Establish a patient registry and natural history study database;

- Advance patient-focused discovery and pre-clinical research and development;

- Work with NIH and the Food and Drug Administration (FDA); and

- Assist with post-market surveillance.

A Therapy Development Road Map

The Toolkit is organized into five areas to address where users may be in the development process:

- **Getting Started** — Describes how therapies are developed, how to prioritize activities, why patient involvement is important and how to build relationships with other stakeholders.

- **Discovery** — Explains how potential therapies often are discovered, how to participate early in the discovery and development process, and how to "grow your field" of research interest through collaborations, patient registries and natural history study databases, funding sources and translational tools.

- **Preparing for Clinical Trials** — Describes the testing process and how patient groups can collaborate with researchers and industry partners on study design, preparation and participation.

- **Clinical Trials and FDA Review** — Provides tools that can help patients connect to clinical trials and take part in the FDA regulatory review process.

- **After FDA Approval** — Provides help with integrating new treatments into clinical care.

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Creating the Toolkit

Launched in September 2017, the Toolkit includes resources that have been developed primarily for the rare diseases community to facilitate therapeutics research and development. Since early 2016, NCATS has worked with a diverse group of partners in the rare diseases community to conduct an extensive landscape analysis of available tools. These resources were defined, characterized and organized in a centralized portal that can be helpful to all patient groups regardless of how far along in the research and development process they might be.

Toolkit co-chairs Ronald J. Bartek, co-founder of the Friedreich's Ataxia Research Alliance (FARA), and Annie Kennedy, senior vice president of legislation and public policy for Parent Project Muscular Dystrophy, collaborated with NCATS and a diverse range of patient groups as part of a working group for nearly two years to identify and evaluate existing resources.

Developed by the rare diseases community to facilitate therapeutics research and development, the Toolkit offers many tools that may be useful to patient advocacy groups for both rare and common diseases and conditions. The working group thoroughly evaluated the tools to make sure they met the team's criteria of being useable, accessible and practical. The group also identified gaps in the types of tools available and discussed how those gaps could be addressed. Patients and patient advocates helped design and test the Toolkit website for usability and functionality.

NCATS will continue to partner with the patient community to add new tools; ensure that existing content is accurate, timely and relevant; and identify gaps so that new tools can be developed.

For questions about the Toolkit or suggestions for improvement, please use the online form.

For More Information

About the Toolkit
ncats.nih.gov/toolkit

Go to the Toolkit
rarediseases.info.nih.gov/toolkit

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ncats.nih.gov

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