



National Center
for Advancing
Translational Sciences

NCATS Toolkit for Patient-Focused Therapy Development

Online resources for patient groups to
advance medical research for rare diseases.



What the NCATS Toolkit offers:

- Information on how to build relationships with partners in academia, government, and industry.
- Tools to help you prioritize your activities throughout the stages of therapy development.
- Strategies to ensure that patients are engaged as essential partners from beginning to end of the research and development process.

<https://rarediseases.info.nih.gov/toolkit>

How can the NCATS Toolkit help?

Getting Started

- Learn about how therapies are developed
- Understand the importance of patient involvement in research
- Build relationships with key stakeholders

Discovery

- Discover funding resources to support research
- Identify translational tools needed to advance research

Preparing for Clinical Trials

- Understand the importance of natural history studies
- Get involved in the clinical trial design process

Clinical Trials and FDA Review

- Discover ways to increase patient participation in clinical trials
- Understand how to participate in FDA review

After FDA Approval

- Find tools for integrating new therapies into clinical care
- Develop programs to speed diagnosis
- Create therapy and care guidelines
- Identify barriers to reimbursement



Get the information you need!

Visit the NCATS Toolkit website:

<https://rarediseases.info.nih.gov/toolkit>

Share your feedback

The NCATS Toolkit was developed in partnership with the rare diseases community. Please contact us to share your stories, suggestions, successes, and challenges via our online form:

<https://rarediseases.info.nih.gov/toolkit/about#contact-us>