Invest in Innovation: Support Funding for the NIH and FDA

CYSTIC FIBROSIS FOUNDATION

The National Institutes of Health (NIH) and the Food and Drug Administration (FDA) play a critical role in advancing therapies for cystic fibrosis and other rare diseases.

Robust funding for the NATIONAL INSTITUTES OF HEALTH helps ensure innovation in basic research and a full pipeline of cystic fibrosis (CF) therapies.

- NIH-funded research fuels the development of new treatments and lays the groundwork for critical new therapies for those with CF.
- NIH provides critical resources for recruiting the next generation of researchers and retaining a strong scientific workforce.

NIH directly supports nearly 380,000 JOBS and $65 BILLION in economic activity across the United States.

Drug approvals by the FDA reached a new high in 2017, with 46 NEW DRUGS APPROVED. More than 400 RARE DISEASE DRUGS AND BIOLOGICS have been approved in the last 30 years.

A well-resourced FOOD AND DRUG ADMINISTRATION helps advance new CF therapies and ensures they are safe and effective.

- The FDA's guidance and expertise is critical in overcoming barriers to developing and evaluating treatments for rare diseases.
- The FDA evaluated and approved two groundbreaking therapies to treat the underlying genetic cause of CF in record time using accelerated review pathways.
- In 2017, the FDA developed a novel approach to expand the use of Kalydeco® for certain rare CF mutations using laboratory data when a full clinical trial was not viable, saving both time and resources.
- Robust funding ensures the FDA has enough staff to thoroughly and efficiently evaluate new drugs for CF and other rare and life-threatening diseases.

The CYSTIC FIBROSIS FOUNDATION builds on the work of the NIH and FDA to provide additional resources for each stage of the research process.

This collaborative approach is cost-efficient and yields new insights to advance CF treatments and care. **To date, 13 CF therapies have been approved, including three that treat the underlying cause of the disease.**

EARLY-STAGE BASIC RESEARCH FOR CF

– Understanding the CF gene

– CF disease models

CF DRUG DEVELOPMENT

– Clinical trials conducted in the Cystic Fibrosis Foundation Therapeutics Development Network

– Developing gene editing techniques

CLINICAL CARE

– Research grants funded through CF Lung Transplant Initiative

– Comparative effectiveness research

HELP US CONTINUE THIS EXTRAORDINARY PROGRESS.

SUPPORT ROBUST FUNDING FOR NIH AND FDA IN FY 2019.

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