PHILANTHROPY ADVISORY SERVICE
MILKEN INSTITUTE

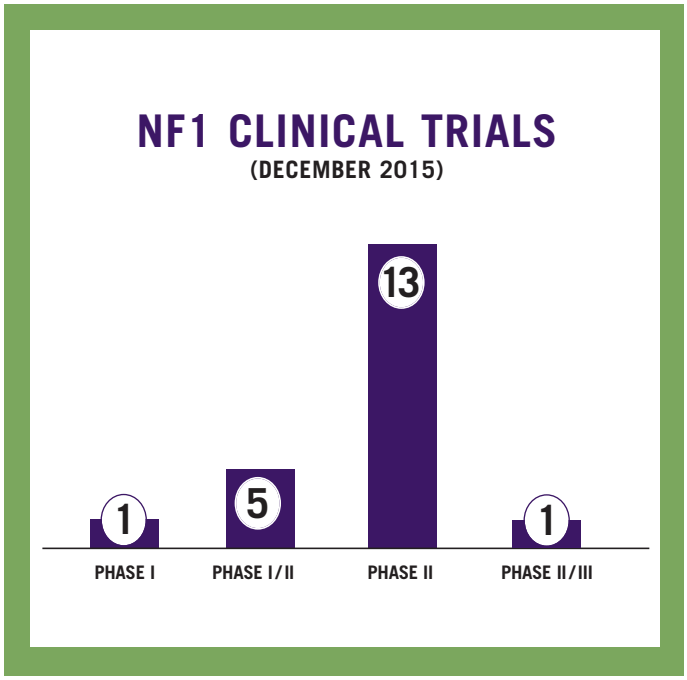
Neurofibromatosis Type 1

A GIVING SMARTER GUIDE TO ACCELERATING RESEARCH PROGRESS

An Executive Summary

NEUROFIBROMATOSIS TYPE 1 (NF1) is a genetic disease that can cause a broad range of abnormalities all throughout the body. A hallmark feature of the disease is the growth of tumors on nerves, which vary in size and number. Depending on their location in the body, the tumors can compromise essential functions such as vision or can result in physical disfigurement. As a developmental disorder, NF1 can also cause cognitive disability, skeletal deformities, and cardiovascular malfunction. While symptoms appear early in life, they may get worse over time or new ones may arise. **The course of the disease is both unpredictable and variable among individuals.** Even within the same family, patients may experience different symptoms at varying degrees of severity.

In the United States, there are roughly 100,000 individuals living with the disease. Though it is classified as a rare



To date, there are no effective therapies to reverse disease symptoms or to prevent new ones from arising.

THERE IS NO KNOWN CURE FOR NF1.

NF1 is one of the most common genetic diseases, affecting approximately 100,000 individuals in the United States. It is more prevalent than cystic fibrosis, Duchenne muscular dystrophy, and Huntington's disease combined.

NF1 EQUALLY AFFECTS ALL GENDERS, RACES, AND ETHNICITIES.

disease, NF1 is more prevalent than cystic fibrosis, Duchenne muscular dystrophy, and Huntington's disease combined. It equally affects all genders, races, and ethnicities.

To date, there are no effective therapies to reverse disease symptoms or to prevent new ones from arising. There is no cure for NF1. Research and development in the field is hampered by several challenges, including:

- A highly-variable clinical care setting
- Limited access to patient tissue samples for clinical research
- Lack of biomarker discovery and development efforts
- Limited understanding of the underlying disease biology

Strategic investments in infrastructure for standardized clinical care and large-scale collection of patient data would enable high-quality care, increase knowledge about the disease, and accelerate therapeutic development.

The Milken Institute Philanthropy Advisory Service has developed this Giving Smarter Guide for NF1 to help patients, supporters, and other stakeholders understand the state of the science and make informed decisions when directing their philanthropic investments. Readers will be able to use this guide to pinpoint research solutions aligned with their interests. The Giving Smarter Guide will help answer the following questions:

- What are key facts about NF1?
- What is the current state of care?
- What is the current state of research?
- What are major barriers to progress?
- Why should I invest in NF1 research?
- How can philanthropy advance new therapies for NF1?

FOR THE FULL GIVING SMARTER GUIDE, VISIT PHILANTHROPY.MILKENINSTITUTE.ORG/REPORTS

The Philanthropy Advisory Service, a program of the Milken Institute, counsels philanthropists, family offices, wealth advisors, and foundations seeking to make transformative investments in medical research. We provide comprehensive, digestible information that helps philanthropists evaluate research efforts and funding opportunities in various disease areas. Our analysis is shaped by scientific advisory boards, in-depth due diligence, and an objective framework for evaluation.
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