FD/MAS ALLIANCE

RESEARCH. ADVOCATE. COLLABORATE.

FD/MAS Research at Risk: Funding Cuts Threaten Critical Studies

The FD/MAS community urges policymakers and stakeholders to recognize the **critical need for continued NIH/NIDCR/ ClinicalTrials.gov ID: NCT00001727 funding**. Without sustained support, decades of progress in bone and endocrine disease research could be lost.

MEDICAL RESEARCH IS AN ECONOMIC ISSUE

- Every $1 invested in NIH research generates $2.64 in economic activity
- The average financial strain on families with a rare disease diagnosis was $80,436 in 2019. Finding effective therapies reduces healthcare costs and prevents complications.
- The National Institute of Dental and Craniofacial Research (NIDCR) budget has seen minimal growth over the past few years. It is approximately .00848% of the federal budget.

This is why NIH funding has always had strong bipartisan support. ***Medical research drives innovation, supports jobs, and strengthens the U.S. as a global scientific leader. Now is the time to keep America at the cutting edge of research and health.***

NIH-Funded FD/MAS Research

Ongoing natural history study of fibrous dysplasia, McCune-Albright syndrome (FD/MAS) a rare bone and endocrine disease

Over 300 participants, ages 1 to 102 years old followed since 1998

Improved therapies and insights into bone biology that help the broader scientific and medical communities including skeletal fractures, osteoarthritis, and osteoporosis

Why Study Rare Disease?

Rare disease is not rare; **10% of Americans have a rare disease**

Rare genetic mutations act as natural experiments, helping researchers uncover fundamental biological mechanisms

Breakthroughs in FD/MAS and other rare conditions lead to advances in treatments for other diseases by providing unique insights into how the human body works

The Screening and Natural History of FD/MAS Major Accomplishments

- Discovered that fibrous dysplasia (FD) is a disease of skeletal stem cells, and made major insights into skeletal stem cell biology
- Discovered that bone cells are the source of the phosphate regulating hormone FGF23, and defined many aspects of FGF23 biology and physiology
- Discovered that overexpression of RANKL by FD cells is a driver of FD pathophysiology and that the anti-RANKL drug denosumab is a promising treatment for FD
- In public-private partnerships with industry, conducted clinical trials with pegvisomant, letrozole, denosumab and burosumab, that will change the care of the disease and inform the use of these drugs in other diseases.