



NORD®
National Organization
for Rare Disorders

Rare Can Be Everywhere: Finding Community When Your Population is Small

Neena Nizar, Founder and President, the Jansen Foundation
Julia Vitarello, Founder and Chief Executive Mila's Miracle
Foundation



www.thejanssensfoundation.org

Dr. Harald Jueppner had been researching Jansen's disease for 20 years, but *never* met a patient.



Rare Disease Day
2016, NIH



A.I. Dupont Hospital
2016, Delaware

The Jansen's Foundation Mission

The Jansen's Foundations goal is to speed up research process, obtain all the necessary approvals, and to start the first testing of a disease-modifying peptide in an adult patient with Jansen's disease.

- Nov. 2017 – RO1 NIH grant – pre-clinical studies
- June 2018 – Pre-IND meeting with FDA
- Sept 2018 – Patient Registry

2019 –
NIH TRND
FUNDING





- There's not only ONE pathway to cures
- Find Your Research Team – A-Team
- Engage with the FDA
- Gather data – Natural History Study
- Find measurable outcomes
- Reach out to the NIH
- Always plan for next steps
- Share and collaborate



Warriors of Hope!