

RESEARCH UPDATE SESSION

NTSAD ANNUAL FAMILY CONFERENCE
APRIL 13, 2018

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THE MATHEW FORBES ROMER FOUNDATION
LYSOGENE

NTSAD'S RESEARCH INITIATIVE

- The Research Initiative started in 2002 with the goal to focus and accelerate research by collaborating with scientists, clinicians, and other patient organizations.
- NTSAD has funded 61 projects in the Research Initiative (RI) and another 13 projects through the Lysosomal Storage Disease Research Consortium (LSDRC).
- Close to \$4 million in grants have been awarded for RI projects in the last 15 years. The impact of these grants has grown as several grantees have collectively received over \$21 million in funding from NIH.
- Funding in line with NTSAD's research strategy to lead efforts and fund work in a number of areas...

NTSAD'S RESEARCH STRATEGY

- Seed grants for innovative therapeutic approaches
- Gene therapies
- Clinical trial readiness

NTSAD'S RESEARCH STRATEGY

- Seed grants for innovative therapeutic approaches
 - Novel therapeutic approaches
 - Small molecule approaches
 - Intranasal therapy
 - Development of cell and animal models
- Gene therapies
- Clinical trial readiness

NTSAD'S RESEARCH STRATEGY

- Seed grants for innovative therapeutic approaches
- Gene therapies
 - TSGT consortium
 - Canavan gene therapy
 - Intracranial and intravenous approaches
 - Combination gene therapy/stem cell therapy approaches
- Clinical trial readiness

NTSAD'S RESEARCH STRATEGY

- Seed grants for innovative therapeutic approaches
- Gene therapies
- Clinical trial readiness
 - Biomarker studies
 - Natural history studies
 - Wearable technologies
 - Newborn screening pilot
 - Genetic testing variant study

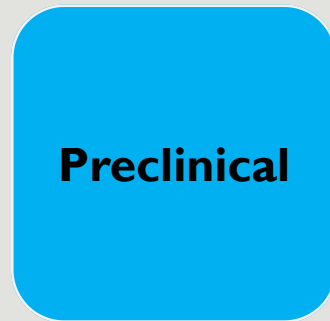
REQUEST FOR PROPOSAL GUIDELINES AND PROCESS

- Research can be basic research, translational studies, clinical studies, clinical trial readiness
- Two part application process
 - Pre-application review
 - Full application for selected studies
- Grants reviewed for scientific merit, feasibility, likelihood of success, unmet needs

NTSAD SUPPORTS ALL STAGES OF DRUG DEVELOPMENT PROCESS



Small Molecules
Novel approaches
to delivery



Development of
models



Biomarkers
Natural History
studies



NTSAD Annual Family Conference Research Update Agenda

9:05-9:20 Welcome & Introduction (Staci Kallish, DO)

9:20-10:00 Keynote address (Steven Walkley, PhD, DVM)
Neurometabolic Disorders – A New Era is Upon Us

10:00-10:20 Involving Patients in Research (Rick Karl, Sue Kahn)
Patient Insight Networks (PINs)
TREND

10:20-10:40 Preparing for Clinical Trials (Staci Kallish, DO, Cynthia Tift, MD, PhD)

10:40-11:00 Break

11:00-12:00 Small groups

Canavan disease (Dominic Gessler)

GM1 (Samantha Parker, Doug Martin, PhD, Alessandra D'Azzo, PhD)

Infantile/juvenile GM2 (Miguel Sena-Esteves, PhD, Cynthia Tift, MD, PhD)

Late-onset GM2 (Camilo Toro, MD, Sarah Ying, MD, Heather Grey-Edwards, DVM,

PhD)