Micro Grant Application Form

The Wiedemann-Steiner Syndrome Foundation (WSSF) believes that lack of research presents the biggest barrier to care for patients. Two awards of up to $7,500 USD are available in 2018 to expedite the exploration of key issues and facilitate research that can impact patient care and medical practice in the following fields:

1. Neuropsychological profile

Patients with Wiedemann-Steiner syndrome (WSS) typically present with some degree of developmental delay and mutations of the MLL1/KMT2A gene have a recognized relationship to autism. The literature and patient reports highlight additional cognitive challenges including:

- Aggressive behavior
- Anxiety
- Attention Deficit Hyperactivity Disorder
- Executive functioning deficits (e.g. working memory, impulse control)

- Intellectual disability
- Learning disability
- Motor delays
- Speech and language delays

Priorities for parents include:

- Understanding the neuropsychological profile and the recommended therapies, educational strategies, accommodations, and behavior management approaches to help patients reach their highest potential.

- Understanding the range and prevalence of intellectual disability, Autism Spectrum Disorder, Attention Deficit Hyperactivity Disorder, and anxiety disorder in the WSS population.

- Understanding the range of adaptive functioning and degree of independence in adulthood.

2. Feeding/digestive issues

Most WSS patients experience feeding difficulties and failure to thrive in infancy and tube feeding is common. Functional gastrointestinal disorders are a hallmark of WSS. Constipation, motility disorders, bowel obstruction, and reflux are often severe, leading to total parental nutrition (TPN) in rare cases.

Priorities for parents include:

- Developing a WSS growth chart, documenting typical growth profile with and without tube feeding.

- Understanding the prevalence and range of severity of gastrointestinal disorders in the WSS population with recommended screening and diagnostic workup.

- Best practices for feeding the WSS infant/toddler (oral and tube feeding).

- Understanding the full range of effective treatments, including complementary and alternative therapies, for motility disorders.

ABOUT WIEDEMANN-STEINER SYNDROME

Wiedemann-Steiner syndrome (WSS) is a rare genetic disorder resulting from mutations in the MLL (also known as KMT2A) gene on the long arm of chromosome 11. The gene encodes a histone-modification enzyme forming part of the epigenetic machinery that helps modify the expression of other genes. The condition is autosomal dominant and in the majority of cases to date, the mutation occurred de novo.

Our active patient community demonstrates a wide spectrum of presentations, from profound to mild disability. WSS may be related to global developmental delays, sleeping difficulties, feeding and digestion complexities, atypical facial features, short/petite stature, hypotonia, dental issues, hairy elbows, etc.

The Wiedemann-Steiner Syndrome Foundation (WSSF) has established a patient registry in partnership with the Coordination of Rare Diseases at Sanford (CoRDS), an international registry program for individuals with rare diseases. The Foundation is committed to sharing available data with researchers and will promote studies and help recruit subjects for micro grant funded projects.

AWARD PROCESS

Awards will be in effect for a maximum of one year from project start date and applicants should ask only for what is needed to complete the proposed study as funds are limited. Travel expenses to conferences will not be covered but supplies, personnel, technical/ software, etc. will be. No overhead is paid with a micro grant.

PROJECT CRITERIA

- Involve Wiedemann-Steiner Syndrome
- Directly impact patient care for one or more patients
- Scientifically valid and feasible
- Research ethics board approval, where applicable
- Studies that involve collaborations between more than one research institution will receive special consideration

APPLICATION REQUIREMENTS

Research proposals must include:

- How the study will improve care
- Scientific summary containing background, hypothesis, methods
- Budget justification
- Lay summary addressing a non-medical, non-scientific audience
- Additional sources of funding and rationale for requesting a grant from this program

REVIEW AND SELECTION PROCESS

Applications will be reviewed by both the scientific advisors and parent (lay) board members of the Wiedemann-Steiner Syndrome Foundation for their impact on care, scientific merit, and relevance to the priorities of patients and their families. Applications are being collected on a rolling basis in 2018. Upon submission of your application, you can expect a response via e-mail and/or mail within 45 days.

ENQUIRIES

Please contact the program coordinator, Libby Woolford, at WSSFoundation@gmail.com or 916-502-2120

REPORTING GUIDELINES

- Grantees are required to submit a final report at the completion of the award (12 months) summarizing major activities and research findings, with a lay summary outlining how their work improves patient care.

- An oral presentation to parents is required, with options to join the WSSF annual conference or present through online video-conference.

- Funded projects are expected to lead to dissemination of results through publication, meeting presentation, and/or generate preliminary data for larger studies directed at improving patient care.

- Grantees will notify the WSSF if other grants are received as a result of micro grant funding.

- Grantees will acknowledge the WSSF in publications resulting from micro grant support and will notify the WSSF when such papers have been submitted.

- WSSF will seek non-exclusive permission to publish final reports and/or published research results on the Foundation's website.

APPLICATION FORMAT

Applications should be submitted by email to WSSFoundation@gmail.com using the template provided below. Applicants will receive a confirmation from the Foundation when their application has been received.

Micro Grant Application Form

1. Category of micro grant requested

Neuropsychological profile ☐

Feeding/digestive issues ☐

2. Applicant Information

Applicant Name

Email

Phone

Institution Name

Address

Co-applicant Name

Email

Phone

Institution Name

Address

Institution supervisor who may receive funds

Name

Email

Phone

Institutional financial contact

Name

Email

Phone

3. Project Information

Project title

Lay Language Project Summary

This will be how our parent reviewers will assess your application. If approved, this section will be posted online so please do not include any proprietary or patient-identifying information. (Max 2000 characters)

Scientific Summary

This is how our scientific/medical reviewers will assess your application (Max 3000 characters)

Citations

Total Funds Requested $(USD)

Please only ask for what you need. Maximum request is currently $7,500 USD

4. Budget

Include additional grants or donations supporting this research project.

5. Applicant's Curriculum Vitae

Attach a current curriculum vitae for primary researchers.

6. Terms

By submitting an application, I agree to

- Submit a final report at the completion of the award (February 28th, 2019) summarizing major activities and research findings, with a lay summary outlining how the work improves patient care.

- Present to parents, with options to join the WSSF annual conference or present through online video-conference.

- Notify the WSSF if other grants are received as a result of micro grant funding.

- Acknowledge the WSSF in publications resulting from micro grant support and notify the WSSF when such papers have been submitted.

- Provide WSSF non-exclusive permission to publish the final report and/or published research results on the Foundation's website.

- Submit a research ethics board approval certificate, where applicable.