Associazione Italiana Sindrome di Shwachman-Diamond (AISS)
Via Pioveghetto 15, - 35136 Padova
Tel - FAX +39 049 8736130
E-mail: aiss@shwachman.it

Shwachman-Diamond Syndrome Italian Research Grant

Maximum Amount euro 10.000,00

Firm Deadline for Receipt of Applications: 31 March 2012

Eligibility: Persons applying for these grants if not in a faculty position need to provide a declaration by a supervisor with a position in the department (not a training position) and with authority to hold an independent research grant.

Terms of Support: Support may be provided for one (1) year in an amount not to exceed E 10,000. Indirect costs are permitted and are not to exceed 10% of the total costs.
The AISS will provide preference to those applications in which funds are used for supplies, equipment, technicians and other expenses and not for support of the salary of the PI or co-PIs.

Review: All applications will be reviewed by the AISS Scientific Committee (AISS-SC) or its designees.

Application: The application contains two sections.

Section 1, forms attached. The applicant and co-applicants must also include a current curriculum vitae. Section 2: Research Plan, divided as indicated below. Parts A through D should not exceed 6 pages, using a font no smaller than 10 point.

Part A. Specific aims
Part B. Significance and background
Part C. Preliminary studies
Part D. Experimental design and methods
Part E. References (not included in the 6 page limit)
Part F. Relevance of the research to Shwachman-Diamond Syndrome
Part G. For junior faculty separate letter from supervisor or department head confirming commitment to project, and to provision of space and facilities
Part K. If human subjects and animals are involved, a statement by the PI or supervisor overseeing human or animal studies is compulsory. If considered as necessary by the AISS-SC, more information about ethical committee study approval may be asked.

Submission by email to the AISS: aiss@shwachman.it

1. Title of Proposal: **NEW REFERENCE GROWTH CHARTS FOR ITALIAN PATIENTS WITH SHWACHMAN-DIAMOND SYNDROME**

2**. Applicant Information:**

Name: _GLORIA TRIDELLO_____

Title and Degree(s): Biostatistician, MSc.
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Work Address: _CYSTIC FIBROSIS CENTER, AZIENDA OSPEDALIERA UNIVERSITARIA INTEGRATA DI VERONA, P.zle STEFANI 1, 37125, VERONA

Telephone: +39.045.812.7216_____
FAX: +39.045.812.2042 _____

Email: gloria.tridello@ospedaleuniverona.it_____

3. **Applicant Curriculum Vitae**: beginning on the next page, with 2 page limit. This will form application pages 3 and 4.

PERSONAL INFORMATION

Name:	**Tridello, Gloria**
Date of Birth:	**14th July 1980**
Place of Birth:	**Padua**
Nationality and Citizenship:	**Italian**
Home Address:	**23 via Orazio, Montegrotto Terme, Padua**
Tel:	**(Mobile): +39 349 5002437**
E-mail address:	**gloria.tridello@ospedaleuniverona.it**

PRESENT POSITION

From April 2011:
Biostatistician at
Centro Fibrosi Cistica
Azienda Ospedaliera Universitaria Integrata di Verona
piazzale Stefani, 1
And at
U.O.C. Oncoematologia Pediatrica
Piazzale L.A. Scuro, 10 - Policlinico G.B. Rossi

- design and analysis of observational studies and clinical trials

PAST POSITIONS

From April 2009 to March 2011
Biostatistician at
European Organisation for Research and Treatment of Cancer – EORTC
Avenue E. Mounier, 83/11, 1200 Brussels, Belgium

- Statistical research on survival analisys
- Phase II and Phase III clinical trial anaysis
- Writing of the final analysis report
- Oral presentation of results
- Quality of life analysis

From January 2003 to March 2009
Statistician at Clinic of Paediatric Haematology-Oncology, University of Padua
Via Giustiniani, 3, 35131 Padova

- Databases creation using Microsoft Access
- Data management for National and International clinical studies
- Data analysis for papers and for oral presentations in National and International meetings
- Statistical data analysis for Medical School graduations, Specialization in Paediatrics and in Oncology, and PhD thesis
- Collaboration in study designs, for example definition of sample size, planning of randomisation list.

PRE-STATISTICAL EDUCATION

Economical oriented High School at “L. B. Alberti” Institute in Abano Terme (Padua), 1994-1999.

STATISTICAL EDUCATION

- 3-year degree (1st cycle degree) course of Statistics at University of Padua (Italy), 1999-2002.
- Achieving of the “Laurea in Statistica e Gestione delle Imprese” (Statistics and Management 1st cycle degree) at the Statistical School of University of Padua (Italy), November, 13th 2002.
- Training period at Operative Centre of Statistical Research – “Fondazione Tettamanti” at University of Milan (review and statistical analysis on data emerging form clinical study), May 2004.
- Master’s level degree (2nd cycle degree) course of Statistics at University of Padua, 2004-2007.
- Achieving the “Laurea Specialistica in Statistica e Informatica” (Statistics and Computing Science Master’s level degree) at the Statistical School of University of Padua (Italy), October, 16th 2007.

Personal skills and competences

Statistical software: R program, S-Plus, SAS, SPSS, Stata.
Microsoft Word, Excel, Access, Power Point, Publisher.

Foreign languages (European level)

English: B2
French: B1

Area of Interest

Study design, databases creation, data analysis, survival analysis, interval censoring analysis.

4. **Applicant's Commitment as Investigator of the Project**:

I agree as the applicant to accept responsibility for the scientific management of this project as outlined in this application. I further agree to submit a report at the end of the granting period.

5**. Applicant's Affirmation**:

I certify that the investigations involving human subjects to be carried out in the application will have approval of the applicant's Institutional Ethical Committee

Approvals from the Institutional Ethical Committee must be included with the application.

6**. Research Results**:

Results of research may be made available to the public through appropriate scientific channels. All publications will bear the statement:

"THIS WORK WAS SUPPORTED BY A GRANT FROM ASSOCIAZIONE ITALIANA SINDROME DI SHWACHMAN-DIAMOND (AISS)"

___ _____ _____31/03/2012_____

Signature of Applicant Date

7**. Applicant's Institution Certification and Commitment:**

I certify that the statements herein and the Applicant's Affirmation are true, complete and accurate to the best of my knowledge and I agree to accept responsibility for the fiscal management of this project as outlined in this application. I further agree to commit this institution to comply with the Associazione Italiana Sindrome di Shwachman-Diamond (AISS) terms and conditions if a grant is awarded as a result of this application.

Name of Institution Official: _PROF. BAROUKH MAURICE ASSAEL

Title: HEAD OF CYSTIC FIBROSIS CENTER

Address: P.ZLE STEFANI 1, 37125, VERONA

Phone: +39.045.812.2370 _____ Fax+39.045.812.2042 _____

E-mail Address: baroukh.assael@ospedaleuniverona.it _____

__ _____31/03/2012_____

Signature of Institution Official Date

ABSTRACT OF RESEARCH PLAN

Within the space provided, summarize the long-term objectives, scientific aims and methodology of the proposal.

TITLE: _ **NEW REFERENCE GROWTH CHARTS FOR ITALIAN PATIENTS WITH SHWACHMAN-DIAMOND SYNDROME**

Shwachman–Diamond syndrome (SDS) is a rare autosomal recessive disorder first described in 1964. The predominant manifestations of the syndrome include exocrine pancreatic insufficiency, bone marrow failure and skeletal abnormalities. Patients frequently present failure to thrive, susceptibility to infections and short stature.
A persistent or intermittent neutropenia occurs in 88–100% of patients. Bone marrow biopsy usually reveals a hypoplastic specimen with varying degrees of hypoplasia and fat infiltration. Some patients may develop myeloblastic syndrome and acute myeloblastic leukemia.
In 2002, researchers from Toronto identified the gene (SBDS) that is altered in SDS on chromosome 7q11; it is expressed ubiquitously in all mammalian tissue. An alteration in learning and behavioral profile is also described, due to the deficits in cognitive abilities.
The negative SGDS gene test does not, however, exclude the diagnosis, and an accurate evaluation of clinical signs is compulsory to diagnose the presence of the syndrome.
Average birth weight is at the 25th percentile. Growth failure with malnutrition is a common feature in the first year of life particularly prior to diagnosis. By the first birthday, over half of patients have dropped below the 3rd percentile for both height and weight. After diagnosis, and with appropriate therapy, most children show normal growth velocity, but remain consistently below the 3rd percentile for height and weight. These alterations are linked directly to the SBDS gene and the growth of these patients differs from the normal children.
Up to date there are no WHO-specific SDS growth charts available. We believe specific charts are a helpful tool in the medical care and for growth surveillance throughout childhood in these patients.
The aim of this retrospective, multicentre, observational study is to estimate the growth chart for patients affected by SDS in order to give a reference tool for evaluating the grow-up of patients with this disease.
All patients of the National Registry of SDS, born between 1975 and 2010, for which the height, weight and the main demographic characteristic are available, will be included in the study. For each patient the following characteristics will be collected: gender, birth date, height, weight, assessment date, available clinic information.
Growth chart will be built in accordance to the LMS method, by Cole and Green, being the primary endpoint the estimation of the percentiles. We estimate to collect about 80 patients.
Inclusion criteria: Patients born between 1975 and 2010 with SDS diagnosis. Patients with at least one measurement per year.
Exclusion criteria: Patients with no enough data available.
The expected duration of the study is 2 years.

BUDGET

version history 26/03/2012

List below a budget by categories for the support. The review committee will carefully consider the appropriateness of your budget. It must be well defined, justified, and realistic to complete the work proposed. The first column defines the total expenses that are expected to be necessary to realistically complete the project. The second column indicates the expenses requested from the AISS. Applicants **will not** be penalized in funding considerations for requiring additional funds beyond what is requested from the Foundation(AISS); however, the true costs of the project must be acknowledged.[This and the section on page 1 re: Other Funding need to be consistent]

EURO Amount Requested for:

	TOTAL COSTS REQUIRED TO COMPLETE PROJECT:	**COSTS REQUESTED FROM AISS**:(not to exceed E 10,000)
Personnel (including fringe benefits): PI: Name: Gloria Tridello Co-I Name: Marco Cipolli Additional personnel (identify role): Name:	6000	4000
Equipment:	1000	1000
Supplies:		
Other Expenses:	1000	1000
Indirect Costs (not to exceed 10% of total)	2600	1300
TOTAL COSTS:	13200	7300

Justification: Define and justify expenses in each category. Explain the role of each of the individuals named in the Personnel section. The justification must include an explanation of what each category contributes to the project. Also explain any marked differences between the first- and second-year expenses in a particular category. The AISS will provide preference to those applications in which funds are used for supplies, equipment, technicians and other expenses and not for support of the salary of the PI or co-PIs. The AISS-SC may ask for further expense details.

Equipment: A computer will be necessary during all the steps of the project, being the data analysis the main activity.

Other Expenses: Since that the study is multicenter, in order to finalize the data collection, some trips to the different centers need to be planned, thus there will be some travel expenses.

Other expenses:

Indirect Costs: A statistical software will be used to perform analysis. The cost for this software is 2600 euro per year.

Other Support for this Project:

Applicants are allowed to receive funding from other sources for parts of the project not funded by the AISS. Please, list all other funding sources.

No other funding will be received for this project

Research Plan