

SMA Europe

European Call for Projects 2009

Contents:

1. Executive summary
2. Scientific Advisory Board (SAB)
3. Application package and procedures

1. Executive summary

At the meeting of SMA Europe in January 2009 it was agreed that AFM (France), VSN (Netherlands), DGM-SMA (Germany), Famiglie SMA (Italy), the Jennifer Trust for SMA (UK), the SMA Trust (UK), and Fundame (Spain) (henceforth, also called ‘partner organizations’ of the SMA-EUROPE association) will launch a second joint call for projects, aimed at funding international research for finding a cure to SMA. This follows the successful conclusion of our first Call for Projects in 2008.

The call for projects will be given adequate publicity. The screening of the projects will be provided by the same high-level Scientific Advisory Board as in 2008, through a thorough peer-review process.

The composition of the Scientific Advisory Board (SAB) is reported in section 2. The application package and procedures are described in Section 3.

Final decisions on projects to be funded will be taken — based on the recommendations of the SAB — by the representatives of the partner organizations, at joint meetings. Each selected project may be funded by one or more partner organisations. Contractual responsibility for any one project will be taken directly by the specific partner organisation(s) funding the project. Intellectual property rights, if relevant, will be negotiated on an individual project basis between the partner organisation(s) and the project’s investigators.

The partner organisations involved in this call include:

- AFM (France)
- DGM-SMA (Germany)
- Famiglie SMA (Italy)
- Fundame (Spain)
- SMA Trust (UK)
- Jennifer Trust SMA (UK)
- VSN (Netherlands)

Contributions by the partner organisations will ensure that no less than **€500,000** (in addition to those granted in the 2008 Call) is made available to the international SMA research community.

The Call for Projects will be open to any research project aimed to find a cure or therapy for Spinal Muscular Atrophy (SMA). Priority will, however, be given to projects concentrating on the following aspects/fields:

- A. Basic research
- B. Gene and/or Cell therapy
- C. Clinical trials
- D. Biomarkers and/or functional outcome measures
- E. Drug development.

There is a desire for this Call for Projects to both foster **international collaboration** between SMA researchers and to address potential **bottlenecks** in the search for cures and therapies for SMA, and preference may be given to applicants who address these concerns.

Purpose of the SMA Europe call for application

This call will support innovative, interdisciplinary, multi-project approaches to **accelerate cures and therapies for SMA**. The initiative provides a framework for interactions that will reduce the time from concept to product. A multi-institutional partnership approach involving academic, non-profit, and/or commercial/industrial institutions — as well as collaboration between investigators from two or more countries — are encouraged. The creative talents in the required scientific disciplines are not always available in a single institution.

2. Scientific Advisory Board

Members of the Scientific Advisory Board:

1. Enrico Bertini
2. Serge Braun
3. Utz Fischer
4. Eugenio Mercuri
5. Francesco Muntoni (Chairman)
6. Kevin Talbot
7. Eduardo Tizzano
8. Louis Viollet
9. Thomas Voit

The SAB will rely also on external referees with specific expertise relevant to the project. The SAB will abide by an appropriate code of conduct for the choice of external referees, working closely with the AFM Secretariat Permanent.

3. Application package and procedures

The application deadline is **June 15, 2009**.

Two types of research grants will be awarded: **Operating Grants** and **Postdoctoral Fellowships**.

Notification of Awards is anticipated to be made in late 2009 with funding to commence as soon as possible thereafter, subject to contracting between the relevant parties. We recognize that this time line will be affected by the project specifics, notably in the case of potential part or joint funding of a clinical trial. Funds will be provided in Euros in most cases, though the partners retain the right to use other currencies should that be more efficient.

ELIGIBILITY

To be eligible as principal investigator to apply for an **operating grant**, an individual should have the skills, knowledge and resources to carry out the proposed project and coordinate a multi-team project. A principal investigator must:

1. Be a professional or faculty member at an appropriate educational, medical or research institution or SME (small or medium enterprise) biotech and be qualified to conduct and supervise a program of original research;
2. Have access to institutional resources necessary to conduct the proposed research project;
3. Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science or equivalent degree.
4. Have a track record of collaboration with others

To be eligible to apply for a **postdoctoral fellowship**, an applicant must:

1. Be a member of a research team in the laboratory of a senior investigator under whose guidance the applicant will conduct the research project;
2. Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science or equivalent degree.

TERM

Awards are for one year, renewable once. Under exceptional circumstances, grants can be renewed for longer periods of time. Funding for the second year and beyond will depend on receiving a detailed annual report.

AWARDS

Standard operating grants have no specific limitation but usually grants will not exceed €150,000 per year (or equivalent). Grants for part or joint-funding of a clinical trial can be up to a maximum of €500,000 (or equivalent).

Grants for Postdoctoral fellowships must be within standard European norms.

APPLICATION PROCEDURE

The application can be found on www.smaeurope.afm-france.org.

An electronic version of the application is required (**2.0 Mb maximum**, MS Word) and should be e-mailed to sma-europe@afm.genethon.fr

2 copies and 1 original of the complete application should be mailed before June 15, 2009 to:

Madame Claire Legrand-Tabary
SMA-EUROPE
c/o AFM - SP du CS
Bâtiment Risler
Groupe Hospitalier Pitié-Salpêtrière
47-83, Boulevard de l'Hôpital
75651 PARIS CEDEX 13
France

Tél. : 33 (0)1 42 16 96 60

Fax : 33 (0)1 42 16 96 65

APPLICATION GUIDELINES FOR OPERATING GRANTS

1. Must be written in English;
2. Must be accompanied by a list of publications
3. Should be brief and concise (maximum: 16 pages);
4. Should contain the following parts: Lay summary (½ page); scientific summary (½ page); scientific background (2 pages); significance/ relevance to SMA, preliminary data relevant to specific aims (2 pages); specific aims (1 page); experimental rationale and design* including time lines (6 pages); references (2 pages) and a detailed budget in Euros including a detailed budget justification (no page limit).

*For **clinical trials**, the “experimental rationale and design” should encompass pre-clinical data and a detailed clinical study protocol including the number of subjects, location, patient selection criteria, methods of identifying and recruiting patients, description of the informed consent process, the definition of primary and secondary outcomes, the description of statistical methods, the description of the safety monitoring plan and future plan development.

* For **drug development**, a plan should be described for decision-making regarding identification and evaluation of promising agents for therapeutic development (i.e. proof of concept, lead optimisation, etc). If applicable, the role of industrial partners should be described in detail as well as a detailed description of the commercial aspects of the project including IP, patent coverage, licensing, and royalty share issues.

It should be noted that all the documents provided will be treated as strictly confidential

5. Should be accompanied by a CV of the principal investigator and collaborators;
6. Should be accompanied by the agreement of the institution where the research is to take place;
7. Should be accompanied by a detailed list of funding from other agencies for the last five years. Any overlap between existing aims or funding and the current application must be fully explained.
8. If applicable, signed letters of agreement of collaborators(s).

APPLICATION GUIDELINES FOR POSTDOCTORAL FELLOWSHIPS

1. Must be written in English;
2. Must be accompanied by a list of publications;
3. Should be brief and concise (maximum: 9 pages);
4. Should contain the following parts: Lay summary (½ page); scientific summary (½ page); scientific background (1 page); preliminary data relevant to specific aims (1 page); specific aims (1 page); experimental rationale and design including time-lines (3 pages); references (2 pages).
5. Should be accompanied by a CV
6. Should be accompanied by a letter of acceptance by the senior investigator under whose guidance the applicant will carry out the research project. Please describe in detail the scientific environment and support. Location in a department that will provide a stimulating research environment is an additional factor that will be considered in evaluating postdoctoral applicants.
7. Should be accompanied by letters of recommendation from two previous advisors of the applicant.

Please note that the postdoctoral applications will be evaluated in a competitive manner independently from operating grants.

SCORING IN THE EVALUATION PROCESS

Each project will be peer-reviewed according to the following criteria:

1. Relevance and Significance for SMA
2. Project quality / scientific soundness;
3. Innovation;
4. Feasibility (i.e., Investigator, environment, approach);
5. Price to quality ratio – the economic reasonableness of the proposed budget;

1- Relevance and significance for SMA: how is this research relevant to a better understanding of SMA, its causes, cure and/or prevention? Does this study address an important problem? Is there a sufficient body of high quality fundamental, preclinical or clinical research that supports the rationale for the proposed study? What is the potential impact of the proposed intervention on health care and quality of life?

2- Project quality/ Scientific soundness: Are the rationale and experimental design adapted to the study objectives?

3- Innovation: Are the aims of the study original? Does the proposed study design represent advancement in the field? Does the project advance international cooperation in the field?

4- Feasibility: Are the conceptual framework, design, methods, and analyses adequately developed, well integrated, and appropriate to the aims of the project? Does the applicant acknowledge potential problem areas and consider alternative strategies? Is the principal investigator well suited to carry out the proposed study? Is the scientific environment adequate? Is there evidence of institutional support? Have appropriate agreements with collaborators been established?

5- Reasonableness of the budget: Is the budget reasonable in relation to the proposed research?

SELECTION PROCESS

Scores will be given for each category, ranging from 1 to 5, with 1 being the lowest score and 5 the highest score.

The written reviews of referees are an important element in the decision making process, but this is not the sole criterion for deciding the success or failure of an application. In practice, only projects which score highly are likely to be successful. We expect to provide feedback to both successful and unsuccessful applicants in the form of reviewers' comments plus any relevant points arising from the committee discussions, but this may not always be practical. In the case of part/joint funding of clinical trials, applicants may be invited to respond to reviewers' comments before a decision is made by the committee. Whilst there are many inherent problems with the peer review process, we believe that this process constitutes the fairest and most consistent way to assess grant applications. We shall try to ensure that the process is as open and transparent as possible and that we maintain the best possible communications with all our applicants.

March 23, 2009