

# Joint Call for Projects under the auspices of *SMA Europe* €800,000

## 1. Executive Summary

At a meeting of SMA Europe in November 2007 it was agreed that AFM (France), DGM-SMA (Germany), Famiglie SMA (Italy), Fundame (Spain), the SMA Trust (UK) and the Jennifer Trust for SMA (UK) (henceforth, also called ‘partner organizations’) will launch a joint call for projects, aimed at funding research for finding a cure to or therapy for SMA. This is a new initiative and – subject to its success – we hope the first in a series of such calls.

The call for projects will be given adequate publicity and marketing. The screening of the projects will be provided by a high-level Scientific Committee, through a thorough peer-reviewing process. The composition of the Scientific Committee 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 Scientific Committee — 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 organization(s) funding the project. Intellectual property rights, if relevant, would 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)

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: A; **Basic research**; B; **Gene therapy**; C; **Clinical trials**; D; **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, because the creative talents in the required scientific disciplines are rarely available in a single institution, but are not necessary.

## 2. Scientific Board

Members of the Scientific Board:

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

The Scientific Board will rely also on external referees with specific expertise relevant to the project. The scientific board will abide by an appropriate code of conduct for the choice of external referees, working closely with the AFM Secretariat Permanent. External referees will clearly be involved whenever members of the Scientific Board submit a proposal to the call for projects themselves.

## 3. Application package and procedures

The application deadline is **2nd September 2008**.

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

Notification of Awards is anticipated to be made in late 2008 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 more 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).

Amount for Postdoctoral fellowships must be within standard European norms.

## APPLICATION PROCEDURE

The application can be found on [www.smaeurope.afm-france.org](http://www.smaeurope.afm-france.org).

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

9 copies and 2 originals of the complete application should be mailed before September 2, 2008 to:

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 (0.5 page); scientific summary (0.5 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 Euro 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 for 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 optimization, 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 5 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 (1/2 page); scientific summary (1/2 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;
7. Should be accompanied by letters of recommendation from 2 previous advisors of the applicant.

## 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 representing an 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?

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

The written reviews of referees are an important element in the decision making process, but are 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 would 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.