

FONDAZIONE



# Sofinnova Telethon Fund Venture Contest

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## GUIDELINES FOR PREPARING AND SUBMITTING THE FULL APPLICATION ONLINE

### General Instructions

The Application form is available on the **TETRA - Telethon Projects Managements system portal** at <https://projects.telethon.it>.


Applicants are identified as Lead Applicant (in charge of creating and completing the Application) and should pay careful attention to the **Guidelines and instructions**, as **an Application failing to meet the requirements will be rejected**. An accurate Application will facilitate the review process.

Use **English** language only. For abbreviations and acronyms not universally known, spell out the term the first time it is used, with the appropriate abbreviation in parentheses; the abbreviation should then be used thereafter.

The text must be single-spaced, not exceeding the character or word number limitations specified.

**Maximum number of characters in the different sections include spaces.**

### Registration

To register, fill in and submit the Application, refer to the *TETRA Portal Instructions.pdf* - [System Help](#)  available on the Home page.

After the first registration, you can change your email address, if you wish to.

If you have questions concerning the Application, click the **Contact Us** link on the left-hand menu to send a message, or send an email to [TelethonScience@telethon.it](mailto:TelethonScience@telethon.it).

### Personal Details

If you already have a Telethon account, please note that for this round it is not necessary to fully complete your personal profile through the **“Manage my details”** section.

### Application

On the Home page, click My Application on the left menu, and by clicking “New Application” you will find the “Sofinnova Telethon Fund” under “Grant Type” to be selected.

### Completing the Application

The created Application is listed in **My Applications** (link on the left-hand menu of the Home page).

In the **My Applications** home page, on the right, the following button sections are shown:

- **Edit**

Please click on the 'Edit' button if you wish to make any changes to your Application.

- **View/Print**

Please click on the 'View/Print' button to generate the Application form as a PDF file. A PDF of the Application can be downloaded at any time by clicking on this button.

- **Validate**

To validate the Application, click 'Validate'. In the “Validation” section within the Application form eventual errors will be listed.

- **Submit**

The Application form cannot be submitted until it has been validated to ensure that all required fields have been entered, and the data meet the submission requirements. The ‘Submit’ button is active once the Application has been correctly validated.

- **Delete**

If you are sure you wish to delete the Application form, please click on the 'Delete' button. Please note: once an Application has been deleted it cannot be recovered.

To navigate into the Application sections, at the top of the Application page, the following buttons are shown:

**Cancel, Previous, Next, Save, Save and Close.**

Clicking the **Cancel** button all modifications are not saved and you are returned to **My Application** home page. **Previous** and **Next** buttons allow you to navigate into the Application, the **Save** button saves all modifications done, remaining on the Application page you are working on, while clicking on **Save and Close** you can save, exit the Application page and return to the Application form afterwards as often as you like.

**Mandatory fields are indicated by red dots.** To successfully submit an Application, all mandatory fields must be completed. Any items missing before submission are listed in the **Validation** section.

When the Application is validated, the Lead Applicant may **Submit** the Application and a final **Application number** (different from pre-submission reference Number) will be assigned to it.

Please refer to this number in any future communications related to it.

A submitted Application cannot be further modified; should you need to apply some amendments **prior to the Call deadline** date click the **Contact Us** on the left-hand menu.

The Full Application comprises:

- Preliminary information
- General information
- Abstract
- Disease Description and Medical Need
  1. Disease Name
  2. Disease Description and Demographics
  3. Fundamental Biological Change (FBC) and Abnormal Biological Process (ABP)
  4. Morbidity
  5. Available and Foreseen Treatments

- Product Description and Scientific Rationale
  - Therapeutic Product Description
  - Scientific Rationale and Background Data
  - Therapeutic Product Use
  - Technological Approach
- Key Reference
- Cited Literature
- Investment Proposal
  - Aims and Activities
  - Team characteristics
  - Target Disease(s)
  - Budget
  - Current financial support
  - GANNT chart
- Intellectual Property
- Clinical Feasibility (if available)
  - Clinical Feasibility Details
  - References
- Additional Information (if available)
  - IP considerations
  - Manufacturing
  - Regulatory
  - Other
- Team
  - Role of the Scientist(s)
  - Relevant Network
  - Biosketch of the scientist(s)
- Type of Organization
- Declaration
- Validation

## Figures

We strongly encourage the Applicant to limit the number of figures; too many unnecessary figures are not generally appreciated by the reviewers. Do not insert data already published.

The Application form includes one box dedicated to figure uploads, in the Therapeutic Product section, under the “Scientific Rationale Background Data” box.

- All figures and legends must be placed together in one PDF document in A4 format.
- In the Figures PDF, insert a footer with the name of the relevant section of the Application form followed by the indication “Figures” and the page number (for example a PDF uploaded into the Application section “Scientific Rationale and Background data” should have the following footer: “Scientific Rationale and Background data Figures - page 1 of 2”, “Scientific Rationale and Background data Figures - page 2 of 2”, etc.)

- Important notice: in the PDF version of the Application, all Figure files will be automatically collected and displayed at the end of the Application form PDF as an appendix. Make sure that the appropriate figure numbers are correctly indicated in the text.
- Please keep the PDF size below 15 MB, to avoid overloading our servers. Use high resolution pictures only for photographs that require details; in this case a maximum resolution setting of 300 dpi (Photoshop: Image>Image Size>Resolution) for each photo is recommended.
- If you include charts or drawings in your PDF, a resolution of 100 dpi for each picture can be used.

**Make sure all the figures are perfectly legible both on monitor and in print.**

### Preliminary Information - To be submitted by March 26<sup>th</sup>, 2021

Please make sure to fill the following info by March 26<sup>th</sup>, 2021.

#### Project Title

**Grant Total Requested** – The Amount requested should not exceed €1,500,000.00

**Scientist(s) name(s)** – Please provide the name of the scientist(s) who is/are the originator/s of the technology on which the research project is based.

**Brief Description of the proposed Therapeutic Product** (max 500 chars)

**Target disease(s)** – Provide the name of the main target disease and, if available, any other identified target disease.

### General Information

**Lead Applicant** – This field is retrieved from the Registration to the portal.

#### Type of Applicant:

Please choose between Academic Researcher or Start-Up Representative (CEO, founder, etc)

- Academic Researcher
- Start-up Representative

If you're a researcher working in a start-up please choose "Start-up Representative" option. Refer to the "Team" section below to describe your role in the company and your scientific experience.

#### Name of the Organization

- In case of *Academia*, please select your organization from the possible options. If you can't find your organization, please contact us at [telethonscience@telethon.it](mailto:telethonscience@telethon.it).
- In case of a *Start-up*, please insert the name of your company and contact us at [telethonscience@telethon.it](mailto:telethonscience@telethon.it); we will provide the required validation in order to submit the application.

**Suggested Reviewers** - Please indicate one or more names of Clinicians with a strong expertise in the field of the target disease(s).

#### Abstract (Max 2,000 characters)

- Introduce the topic of your work (i.e., description of the Therapeutic Product and which problem it is solving), set it into the broad scientific and biomedical context.
- Describe the recent advancements in the field (i.e., competing products in the market, available treatments) or under preclinical or clinical development (foreseen treatments).
- Comment why the described Therapeutic Product is superior to competing strategies/products.

#### Disease Description and Medical Need (max 12,000 characters)

**1. Disease Name** – Please provide the name of the **main** target disease

In case of genetic disease please provide the following information:

**Disease Codes and Disease OMIM number, ICD-10 code, Orpha Number**, – Provide as many as available for the main target disease.

**Area(s) of Research and Research Type(s)** – Select from the proposed list all the applicable options

**2. Disease Description and Demographics** (max 2,000 characters)

- Describe the main clinical phenotypes of the disease(s).
- Briefly describe (and reference) the demographics of the disease(s), including typical age of onset, calculated annual incidence and prevalence (if any), ethnic distribution (if any), and gender proportions.
- Clearly indicate (and reference) the severity and frequency of specific morbidities of the disease, and timing and frequency of mortality (timing either after disease onset or at patient age), despite use of currently available treatments.
- Describe (and reference) the cause of mortality for the disease.

**3. Fundamental Biological Change (FBC) and Abnormal Biological Process (ABP)** (max 5,000 characters)

- Describe (and reference) what you believe to be the fundamental biological change (FBC) underlying the medical condition – mutation, enzyme/protein change, etc. If there is more than one FBC, please identify and explain.
- Describe (and reference) each abnormal biological process (ABP) stemming directly, or indirectly, from the FBC described. If there is an ABP that you cannot link directly to the FBC, describe (and reference) the indirect linkage to the FBC. If you cannot describe an indirect linkage, then postulate the cause of this unlinked ABP.

**4. Morbidity** (max 1,500 characters)

- Describe, if available, what is known about what level of activity for each ABP is necessary to cause each clinical morbidity (for example, if a morbidity was the result of an enzyme deficiency, what level of enzyme activity would appear to be sufficient to reduce the frequency and/or severity of this specific morbidity) noted above in #2.
- Draw specific links (and reference) between each ABP and each specific morbidity noted above in #2.

**6. Available and Foreseen Treatments** (max 3,500 characters)

- Identify (and reference) currently available treatments and evidence for effectiveness and safety, if any, of each currently available treatment for the condition named in #1 above and for each morbidity named in #4 above and each ABP named in #3 above.
- Provide a general overview on the foreseen treatment(s) within 5 years.

**Product Description and Scientific Rationale** (max 17,000 characters)

**Therapeutic Product Description** (max 1,500 characters)

- Describe the therapeutic product and the technology on which it is based.
- Describe how your proposed product would address each/all ABP and clinical morbidities noted above.

**Scientific Rationale and Background Data** (max 5,000 characters)

- Describe the scientific rationale on which the therapeutic product is based.
- Describe the background data validating the biological target modified/modulated by the therapeutic (possibly including figures)

- Provide the data supporting safety and efficacy of the therapeutic product in a disease model (in vivo or in vitro) (possibly including figures)

**Scientific Rationale and Background Data Figures** – If you have any supporting figure, please upload one .pdf file with a maximum size of 15 Mb. Please use “Scientific Rationale and Background Data Figures” as file name.

**Therapeutic Product Use** (max 4,500 characters)

- Describe how the therapeutic product addresses the unmet medical need.
- Describe and define clearly if the therapeutic product is addressing all patients affected by the specific diseases or a subpopulation and in such a case the relative incidence.
- Describe if and how the therapeutic product can be used for multiple diseases (including non-rare disease indications).
- Describe how the therapeutic product is delivered (and how it could be delivered to human, if available).

**Technological Approach** (max 6,000 characters)

- Describe how the technological approach on which the therapeutic product is based is innovative and overcomes the limits of existing technologies (potential for IP protection, problem being addressed).
- Is it a **platform technology**? If yes explain why it is a platform and which diseases could be addressed through it.
- Indicate if the technological approach on which the therapeutic product is based is amenable to be applied to other therapeutic products intended for other diseases, including non-rare disease indications.

**Competition** (max 3,000 characters)

- Describe how the therapeutic product compares to products available or undergoing pre-clinical and clinical validation for the treatment of the disease.

**Key References** (max 3,500 characters) (\*)

- List 5-10 key references on which your work is based.

**Cited Literature** (max 18,000 characters) (\*)

- List all references.

(\*) The list must include the name of all authors, year of publication, title, book or journal, volume number and page numbers. If a bibliographic management software is being used, the format of the journal “Developmental Dynamics” may be applied.

**Concise references are not allowed.**

The complete list of references will be visible to Reviewers at any evaluation phase.

**Investment Proposal** (max 8,000 characters)

**Aims and Activities** (max 2,000 characters)

- Describe which proof of concept (PoC) will be achieved in a 1 to 3 year time frame and the activities foreseen to reach such PoC and the expected deliverables.

**Team Characteristics** (max 2,000 characters)

- Briefly describe the competences of the team involved, their role in the program, which activities will be performed by the team and which will need to be subcontracted.



**Target disease(s)** (max 2,000 characters)

- Describe which disease(s) shall be addressed by the existing/foreseen Start-up and the rationale for the proposed prioritization.

**Budget** (max 1,000 characters)

- Budget requested to conduct such activities (limited to R&D budget, including subcontracting) – the Applicant will have to state the requested budget up to €1.5 Million and to describe all the items which build up the budget request.

**Current financial support** (max 1,000 characters)

- Please briefly describe your current financial support (research grants, cooperative agreements, contracts, sponsored research agreements, service agreements and/or institutional awards).

**Gantt chart** (pdf file only)

Please upload a GANTT chart describing the timeframe foreseen for the development steps expressed in milestones of the therapeutic product or technological approach.

**Intellectual Property**

Please check all the applicable options.

**1.** The relevant data are included in a patent already filed.

Please fill in the **date of filing**, the **current stage** (e.g., Priority patent application, National patent application, PCT application, Granted Patent...) and the **number of the patent**.

**2.** The relevant data are included in a patent in progress of filing.

**3.** The relevant data are still undisclosed (neither published nor included in any publicly available document, as undergraduate dissertation, PhD thesis, meeting abstract, oral or poster presentation).

**Please note that a requirement to receive the investment is that the existing/foreseen start-up has/will have exclusive rights on the indicated Intellectual Property.**

**Clinical Feasibility – If available**

**This section is recommended but not mandatory.** For those Projects including the Clinical Feasibility, please follow the instructions below. The section must be completed by a clinical expert in the field of the proposed disease.

**Clinical Feasibility Details** (max 6,000 characters)


- Indicate (and reference) if the disease has been addressed by natural history studies.
- Indicate (and reference) if registries have been already established.
- Estimate how many unique patients with this disease have been seen (both primarily and by referral) at your institution or at your clinical reference center during the past 5 years.
- For the unique patients seen at your institution or at your clinical reference center over the past 5 years, estimate how many patients you would consider having mild disease, moderate disease, and severe disease at any time (note the same patient may be mild at some point and severe at another point). Provide the criteria that are used to classify patients as mild, moderate, or severe.
- Indicate (and reference) the existence of validated (either by the clinical community or by a regulatory authority) clinical end points for the proposed indication(s).
- Indicate if specific biomarkers are useful for the indication proposed. For each biomarker that currently exists, describe (and reference) the direct or indirect relationship to each ABP

**References** (max 5,000 characters)

- ### Clinical Feasibility section Instructions

In the *Clinical Expert* box (in the *Clinical Feasibility* section) the Expert's details and the status are shown; in case of error the Expert can be deleted clicking on the **Remove** button.

## Clinical Expert Confirmation and Guidelines

On the left-hand menu of the Home page, click on “My Co-Application” to visualize the Application you’ve been added to and click on the detail icon  to open it.

Reference	Title	Main Applicant	Role	Confirmed	Last Updated	Application Status
[REDACTED]	[REDACTED]	[REDACTED] [REDACTED]	Clinical Expert		[REDACTED] [REDACTED]	Pre-Submission

In the **My Co-Applications** home page, on the right, the following button sections are shown:

- **Edit**  
Please click on the 'Edit' button to open and read the Application. To complete the section you've been invited to, compile please click on the left-hand menu the “Clinical Feasibility” section.
- **View/Print**  
Please click on the 'View/Print' button to generate the Application form as a PDF file. A PDF of the Application can be downloaded at any time by clicking on this button.
- **Validate the Application**  
Please note that this action is not required for the Clinical expert role.
- **Confirm your participation**  
To participate and be able to compile the section, please select the “Confirm” button.



- **Reject your participation**

If you do not wish to participate in this application please click the reject button below. This will send an email informing the lead applicant and will remove you from the application involvement.

To navigate into the Application sections, at the top of the Application page, the following buttons are shown:

**Cancel, Previous, Next, Save, Save and Close.**

Clicking the **Cancel** button all modifications are not saved and you are returned to **My Co-Application** home page. **Previous** and **Next** buttons allow you to navigate into the Application, the **Save** button saves all modifications done, remaining on the Application page you are working on, while clicking on **Save and Close** you can save, exit the Application page and return to the Application form afterwards as often as you like.

### Additional Information – If available

Please fill in the following boxes in case the relative information is available.

**IP considerations** (max 1,000 characters)

- Include details on existing patent, dependence on a particular technology, need to consolidate IP position, co-owners of the IP (if any).

**Manufacturing** (max 1,000 characters)

- Describe the current status of production of the therapeutic product; describe how the therapeutic product is/would be manufactured for laboratory studies, pre-clinical (GLP) studies, and clinical studies (e. g., commercial availability at R&D or clinical grade, contacts with CMOs, preliminary estimations if available).

**Regulatory** (max 1,000 characters)

- Regulatory considerations (e. g., existing orphan drug designation).

**Other** (max 1,000 characters)

- Please add any other relevant information or note.

### Team

**Role of the scientist(s)** (Max 1,000 characters)

Please provide details about specific activities of each scientist of the team and her/his roles in the existing/future start-up.

**Relevant Network (e.g., Clinicians, Medicinal Chemists)** (Max 1,000 characters)

Please indicate any collaboration relevant for the execution of the proposed Project.

**Brief Biosketch of the Scientist(s)** –Please provide the Biosketch for any scientist involved in the project (click on **Add** button for each scientist).

**For each scientist** fill in the required information (fields):

- **First name**
- **Last name**
- **Institution**
- **Department**

- *Please provide a brief description of your career including education, professional appointments, research interests and achievements, indicate 5 recent publications* (max 500 characters)

### Organization Type (max 1,000 characters)

- **Academic Organization**

Briefly describe the academic environment (i.e., number of researchers, relationship with hospitals, universities, etc.)

Or

- **Start-up**

Briefly describe the existing start up (i.e., individual Founders, institutions and investors involved, etc.)

### Declaration

The Applicant has to certify that the information included in the Application is accurate and complete, that he/she is entitled and/or authorized to disclose all the information provided within the Application and that he/she complies with Telethon's terms and conditions, as reported in the "Specific Requirement" section of the Call.

Please duly fill in, sign and upload the Information about Data Processing (see Call Document - Appendix 1).

### Validation

This section will be automatically compiled, once the *Validate* action in the **My Application** Home Page has been done. Please note that the sections highlighted in green are those correctly filled in, while those in red have to be completed and key data are still missing.

### Application submission

The deadline for **online submission is April 23<sup>rd</sup>, 2021 at 1:00 p.m.**

Please consider that clicking the **Submit** button, the Application will be automatically submitted and cannot be further modified.

Before the Submission, download the PDF of your Application to check all the sections; in particular check that all uploaded documents and images are included in the PDF and are clearly legible. Please note that the Applicant is liable for the contents and quality of the Application in its final version.

Fondazione Telethon holds the responsibility and authority in making the final decision on the Application's completeness and eligibility.

March 1<sup>st</sup>, 2021

FONDAZIONE TELETHON