

# **Application Form**

# **Summary**

| Reference number |   |
|------------------|---|
| Institution      |   |
| Lead Applicant   |   |
| Total Requested  | € |

Reference: Telethon\_

| Preliminary Information                                |  |
|--|--|
|  |  |
| 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 based. | o is/are the originator/s of the technology on which the research project is |
|  |  |
| Brief Description of the proposed Thera                | peutic Product   |
|  |  |
|  |  |
| Target disease(s)                                      |  |
|  |  |
|  |  |

Provide the name of the main target disease and, if available, any other identified target disease.

Reference: Telethon\_

## **General Information**

Title Address Line 1
Forename(s) Address Line 2
Surname Address Line 3
Date of Birth County

Nationality Postcode Institution Department

| Type of Applicant |  |
|-------------------|--|
|-------------------|--|

## Name of Academic Organization

Name of Start Up

| Nam | е |  |  |  |  |
|-----|---|--|--|--|--|
|-----|---|--|--|--|--|



| Executive Summary |  |  |  |  |
|-------------------|--|--|--|--|
|                   |  |  |  |  |
|                   |  |  |  |  |
|                   |  |  |  |  |



| <b>Disease Description and Medical Nee</b> | ed  |
|--|---|
|  |   |
| 1. DISEASE NAME                            |   |
| Provide name of main target disease.       |   |
|  |   |
| le the disease genetic and/or rere?        |   |
| Is the disease genetic and/or rare?        |   |
| Genetic Details                            |   |
| Provide as many as possible                |   |
| ORPHA Number                               |   |
|  |   |
| Orphanet classification                    |   |
|  |   |
| OMIM Number                                |   |
|  |   |
| ICD-10 Code                                |   |
|  |   |
| Area(s) of Research                        |   |
| No Area(s) of Research have been added     |   |
| No Alea(s) of Research have been added     |   |
| Decearsh Time                              |   |
| Research Type                              |   |
| No Research Type have been added           |   |
|  |   |
| 2. DISEASE DESCRIPTION and DEMOGR          | APHICS  |
|  |   |
|  |   |
| 3. FUNDAMENTAL BIOLOGICAL CHANG            | E (FBC) AND ABNORMAL BIOLOGICAL PROCESS (ABP) |
|  |   |
|  |   |
| 4. MORBIDITY                               |   |
| 4. WORDINI                                 |   |
|  |   |
|  |   |
| 5. AVAILABLE TREATMENTS and FORES          | SEEN TREATMENTS                               |
|  |   |

| Therapeutic Product and Technological Approach               |  |
|--|--|
|  |  |
| THERAPEUTIC PRODUCT DESCRIPTION                              |  |
|  |  |
|  |  |
| SCIENTIFIC RATIONALE AND BACKGROUND DATA                     |  |
|  |  |
|  |  |
| SCIENTIFIC RATIONALE AND BACKGROUND DATA                     |  |
| supporting documentation Do you have any supporting figures? |  |
| ,                      |  |
|  |  |
| THERAPEUTIC PRODUCT USE                                      |  |
|  |  |
|  |  |
| TECHNOLOGICAL APPROACH                                       |  |
|  |  |

| ( | Competition |
|---|-------------|
|   |             |
|   |             |
|   |             |
|   |             |



## **Key References**

Provide 5-10 key scientific publications on which your work is based



## **Cited Literature**

Please provide Cited Literature



| ivestment Proposal                              |   |
|---|---|
|   |   |
| Aims and Activities                             |   |
|   |   |
|   |   |
|   |   |
| Team characteristics                            |   |
|   |   |
|   |   |
|   |   |
| Target disease(s)                               |   |
| <u> </u>  |   |
|   |   |
|   |   |
| Budget  |   |
|   |   |
|   |   |
|   |   |
| Current financial support                       |   |
|   |   |
|   |   |
|   |   |
| Please upload a GANNT chart (in PDF format) des | scribing the timeframe foreseen for the different Specific Aims and their |

components.

| ntellectual Property                     |  |  |
|--|--|--|
| Please check all the applicable options: |  |  |
| Date of Filing                           |  |  |
| Current Stage                            |  |  |
| Patent number                            |  |  |



#### Clinical Feasibility - (If Available)

#### To be completed by the Clinical Expert identified by the Applicant as expert for the target disease

Is Clinical Feasibility available?

Title

Forename(s)

Surname

Address

Honours Institution Department

Telephone No. Email Address

#### Clinical Feasibility Detail.

- 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 to have 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 above. If there are not
  sufficient biomarkers existing today, propose necessary biomarkers and describe (and reference) the
  direct or indirect relationship to each ABP above.

| References | 5 |  |
|------------|---|--|
|            |   |  |

Reference: Telethon\_

| Additional information - (if Available) |   |  |
|---|---|--|
|   |   |  |
| Is there                                | any additional information you need to add? |  |
|   |   |  |
| IP                                      |   |  |
|   |   |  |
|   |   |  |
| Manufa                                  | cturing                                     |  |
|   |   |  |
|   |   |  |
| Regulat                                 | ory   |  |
|   |   |  |
|   |   |  |
| Other                                   |   |  |
|   |   |  |
|   |   |  |

#### **Team**

| Role of the SCIENTIST(s) |  |
|--------------------------|--|
|                          |  |

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)

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

| Brief Biosketch of the SCIENTIST(s) |           |             |            |   |  |
|-------------------------------------|-----------|-------------|------------|---|--|
| 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 |  |
|                                     |           |             |            |   |  |

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## **Organisation Type**

#### **Academic**

Description of the academic organization (number of researchers, relationship with hospitals, universities)

Start Up.

Description of the existing start up (i.e., individual Founders, institutions and investors involved)



#### **Declaration**

- I hereby certify that all information submitted in the online application form is accurate and complete.
- I hereby certify that I'm entitled and/or authorized to disclose all Information provided within the Application.
- The Applicant declares to have provided any researcher involved in the Application (Key Personnel or Collaborator) with the information attached to the Call for Application in Appendix 1. He/She also declares that the relevant consents have been gathered in order to be authorized to indicate the personal data of any other researcher in the Application.

| Certify   | Not Confirmed |  |  |
|-----------|---------------|--|--|
|           |               |  |  |
| Full Name |               |  |  |
|           |               |  |  |
| Place     |               |  |  |
|           |               |  |  |
| Date      |               |  |  |

Samo

