

# THE TELETHON BOOK

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Facts and Figures about Telethon Research

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2010



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## ABOUT TELETHON ITALY

### WHAT IS TELETHON ITALY

Telethon Italy is the second biggest biomedical charity in Italy. Its mission is to advance biomedical research towards the diagnosis, cure and prevention of muscular dystrophies and other human genetic diseases.

Telethon was founded in 1990 by a patients' association, the [Italian Union for Muscular Dystrophy \(UILDM\)](#), as an initiative inspired by- but not affiliated with- popular Telethon marathons in the USA and France. Telethon Italy focuses on scientific research and does not offer healthcare or material assistance to patients and families. As a part of its mission, Telethon also aims to involve patients and their associations, and the Italian public at large, in the fight against hereditary diseases.

In their first 20 years of activity, Telethon Italy has invested 299.5 million Euros in research and funded 2,200 research projects, ranging from basic research to clinical trials, on more than 440 human genetic diseases. Telethon's research portfolio includes intramural and extramural research, performed in three Telethon institutes and through grants to universities and non-profit research institutes in Italy, respectively.

All funding decisions at Telethon are subject to peer-review and evaluation by an international Scientific Committee and by ad-hoc review panels.

### FIGHTING ALL GENETIC DISEASES THROUGH RESEARCH

#### TELETHON'S MISSION

- We aim to help advance scientific research towards the cure for muscular dystrophy and other genetic diseases.
- We give priority to those genetic diseases which are so rare that they are overlooked by the public and industrial funding agencies.
- We finance outstanding research projects and the finest researchers in Italy.
- We want to involve the Italian population in the fight against genetic diseases.
- We aim to make crystal clear how we spend the money donated to us.

Genetic diseases are caused by alterations in the DNA. Several thousand genetic diseases are known today and their list is constantly growing, due to the discovery of new disease-causing genetic defects. Altogether, genetic disorders involve virtually every organ of the body and affect people of all ages; most of them are still without a definitive cure. Taken individually, genetic diseases are rare, and therefore are neglected by major private and public funding; however, taken as a whole they represent a health and social burden that affects the lives of millions. It is estimated, for example, that eight million babies worldwide are born every year with a severe defect of total or partial genetic origin ([March of Dimes Global Report, 2008](#)).

Research projects on all human hereditary diseases, from basic to clinical, are eligible for Telethon funding, but the priority is given to monogenic diseases which, because of their rarity, are neglected by major public and industrial

funding, as stated in Telethon's mission.

## ORGANIZATION AND MANAGEMENT

Telethon is a non-profit organization recognized by the Italian law, employing a total staff of 446, of which 326 (73.1%) work in its research institutes. Telethon's management is summarized below:

<p><b>Chairperson:</b>  <i>Luca Cordero di Montezemolo</i></p> <p><b>Board of Directors:</b>  <i>Omero Toso (vice-president); Boris Biancheri; Carlo Ferdinando Camacini; Salvatore Di Mauro; Alberto Fontana; Francesca Pasinelli.</i></p> <p><b>Managing Director:</b> <i>Francesca Pasinelli</i></p> <p><b>Director, Administration and Finance:</b> <i>Tiziana Ciracò</i></p> <p><b>Director, Fund Raising:</b> <i>Fulvio Bruno</i></p> <p><b>Chief Scientific Officer:</b> <i>Lucia Monaco</i></p>
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## FUNDRAISING AND ACCOUNTABILITY

Fundraising depends primarily on a solidarity marathon presented on national TV channels every December, and relies strongly on private donations. In 2009 Telethon raised 33.5 million Euros. Transparency, accountability and a high organizational efficiency are major features of Telethon Italy. In the last year, 76% of raised funds were spent on research activities, a rate that is comparable with those of the most efficient charities worldwide (for a comparison see [www.charitynavigator.org](http://www.charitynavigator.org), “program expenses ratio”). Annual financial reports are available at <http://www.telethon.it/chisiamo/download/default.aspx> (in Italian)

## TELETHON AND THE NATIONAL AND INTERNATIONAL RESEARCH COMMUNITY

Telethon is acknowledged as a funding agency promoting excellence and its review process is seen both nationally and internationally as a model of good practice (Pammolli, F., Riccaboni, M., Magazzini, L., CERM report 1-2009, [http://www.cermlab.it/\\_documents/RapportoCERM01\\_2009.pdf](http://www.cermlab.it/_documents/RapportoCERM01_2009.pdf); Jurkat-Rott K, Lehmann-Horn F., Eur J Cell Biol. 2004, 83:93-5).

The world-class quality and competitiveness of Telethon-funded research is also reflected in the number and quality of peer-reviewed publications. The average citation index of Telethon-funded publications is higher than the European and US average in all relevant areas of Telethon research (for details, see the “Achievements of Telethon Research” section further in this document).

The [Telethon Scientific Convention](#), a bi-annual event, displays the state of the art of Telethon-funded research, hosts keynote lectures from internationally renowned speakers and roundtables with patients' associations and gathers all Telethon-funded scientists under one roof. Abstracts of the convention are available online at the [Telethon's website](#).

Telethon grants represent the main funding resource for many productive Italian laboratories involved in the study of genetic diseases that are neglected by public and industrial investments. Telethon is also a

reference for the Italian scientific community at large when it comes to addressing general issues concerning research and peer-review management.

## TELETHON'S RESEARCH PORTFOLIO

With the aim of pursuing the most promising avenues towards the cure of genetic diseases, Telethon has created and maintains a rich and diverse research portfolio that in 2009 included 260 intramural and extramural research projects in progress, encompassing all stages of development, from basic studies to clinical trials.

Extramural projects which Telethon funds through competitive calls, offer more flexibility and therefore allow the choice of the best opportunities towards a cure; intramural research, on the other hand, is funded through non-competitive calls and, although less flexible, offers a greater commitment towards long-term goals and more control in terms of governance and management of intellectual property. In managing its research portfolio, Telethon aims to balance the respective pros and cons of these two models.

Intramural (internal) research is performed in three Telethon institutes: the **Telethon Institute of Genetics and Medicine (TIGEM)**, the **San Raffaele-Telethon Institute for Gene Therapy (HSR-TIGET)**, and the **Dulbecco Telethon Institute (DTI)**. The activities of each institute are detailed further in this document. A fourth intramural institute, **Tecnothon**, was founded in 1994 as a technology laboratory whose mission is to plan and to manufacture functioning prototypes of auxiliary equipment for the physically disabled. Further details on Tecnothon can be found at: <http://www.telethon.it/sites/tecnothon>.

Extramural research is performed in various academic and non-profit institutions in Italy (which are recipients of Telethon external research grants) and encompasses all Telethon's grant-funding activities to external institutes, including research projects, research services/core facilities and program projects.

Currently, Telethon's research portfolio is almost equally balanced between intramural and extramural funding: the overall funding allocated to external and intramural research in the last year is shown in Figure 1.

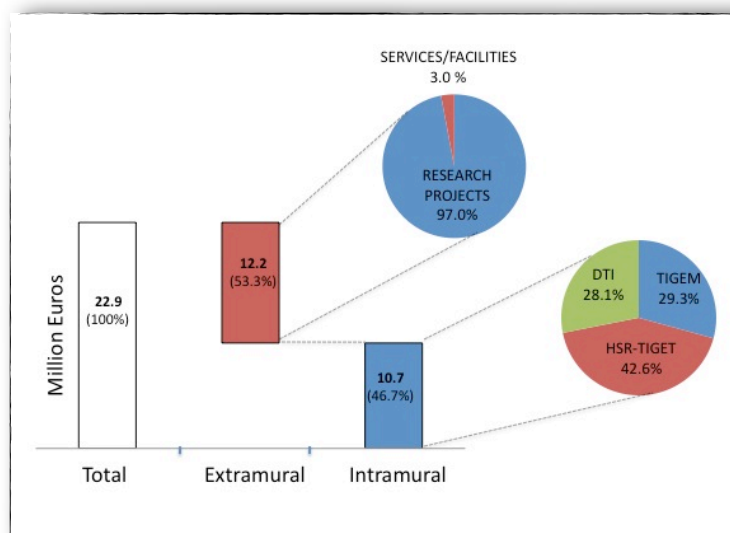


Figure 1. Overall distribution of Telethon funds among extra- and intramural research (July 2008-June 2009) *Source: Telethon.*

## TELETHON CALLS FOR APPLICATIONS

Telethon issues periodic calls for applications for its project grants and for the positions at the Dulbecco-Telethon Institute. All applications are submitted electronically and undergo a peer review selection process (for details please see: “The Peer-Review Process at Telethon” section).

Intramural research projects led by investigators at Telethon’s institutes TIGEM and HSR-TIGET are not currently evaluated within competitive calls for applications; they are however subjected to peer review with the periodical (5-years) re-assessment of the institutes, which entails a site-visit and an ad-hoc review session.

## ELIGIBLE DISEASES AND FUNDING

Telethon funds research projects focusing on diseases of proven genetic origin, while common and multifactorial diseases are not eligible for Telethon funding. Such criteria allow Telethon to focus its efforts towards a cure for monogenic diseases, which are usually rare and therefore are neglected by major public and private fundings despite the heavy toll that they take on patients and their families.

Amyotrophic Lateral Sclerosis (ALS) is excluded from Telethon calls since new dedicated funding opportunities for this disease are available from the “Agenzia di Ricerca per la Sclerosi Laterale Amiotrofica” AriSLA (<http://www.arisla.org/>), a research Agency recently created with Telethon’s support.

Projects aimed at identifying genetic risk factors for complex diseases, along with cancer, multiple sclerosis and acquired immunodeficiencies, are not eligible for Telethon funding.

Overall, Telethon research projects have addressed more than 400 genetic diseases. Figure 2 accounts for the distribution of funding among three major classes of disorders.

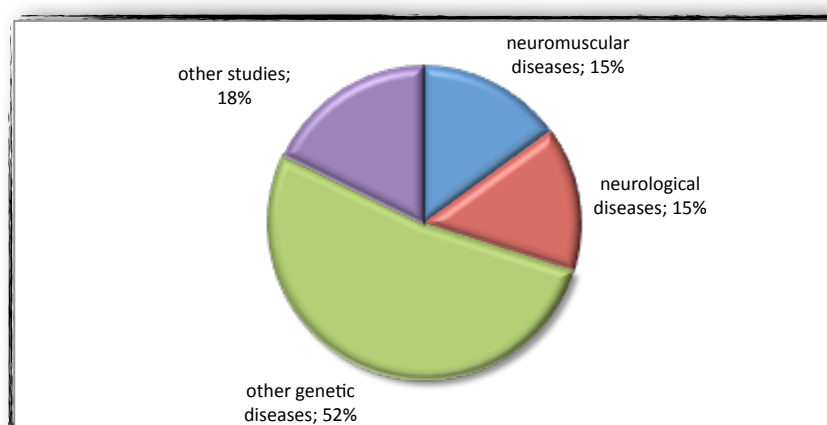


Figure 2. Distribution of Telethon grants among different categories of genetic diseases (July 2008-June 2009). Includes both external and intramural research. The “other studies” category includes basic studies and structural expenses not attributable to a specific class.  
*Source: Telethon.*

## IMPLEMENTING TELETHON RESEARCH STRATEGY

The [Telethon Strategic Plan for Research 2006-2010](#), which was outlined in collaboration with the Telethon Scientific Advisory Board, entails the vision and the priorities of Telethon research and includes keys areas of action regarding, among other things, the management of the project portfolio and the transfer of scientific results into therapies available to patients.

The decision-making process for the implementation of Telethon research strategy is based on the constant monitoring of results and involves the coordinated action of different participants (Figure 3).

**The Scientific Advisory Board** is composed of leaders in different fields of biomedical research that periodically evaluate Telethon progress and offer continuing advice to accomplish the research objectives outlined in the strategic plan.

**The Board of Directors** takes into account the Scientific Advisory Board's recommendations in order to make key decisions regarding Telethon research.

The **Telethon Science Watch Office**, made of professionals reporting to the Chief Scientific Officer, is responsible for monitoring, analyzing and benchmarking Telethon research. The Science Watch Office produces reports on the performance, funding and state of the art of research for the Scientific Advisory Board.

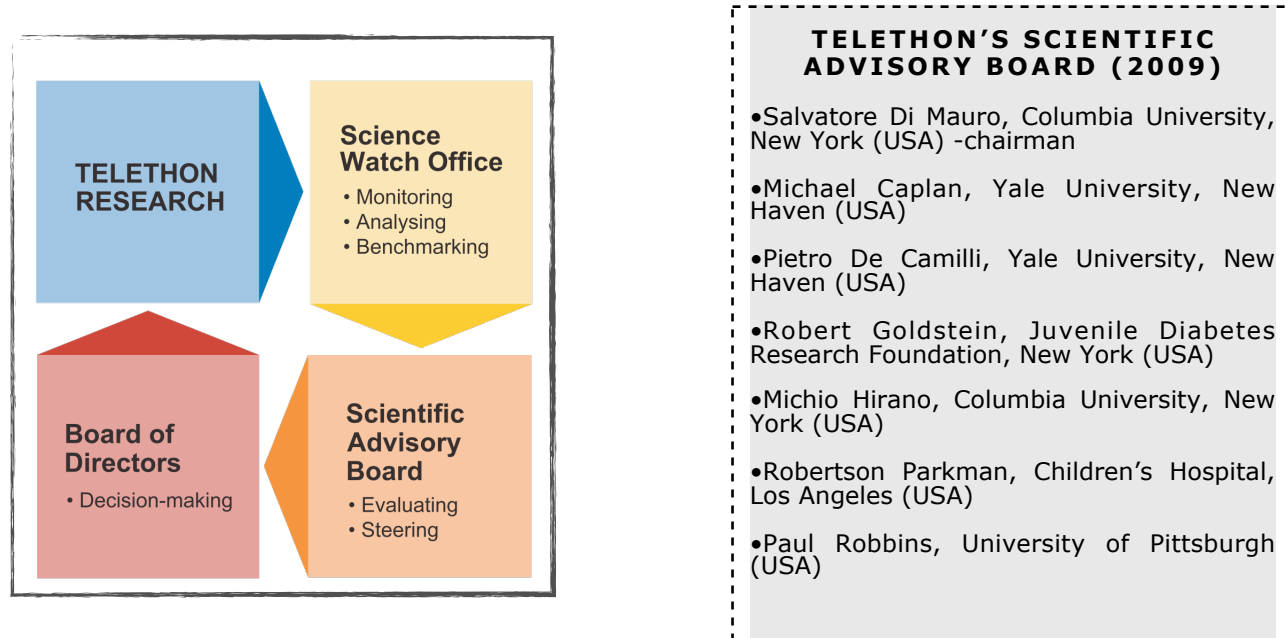


Figure 3. The assessment and decision-making process for implementing research strategy at Telethon.



## TELETHON RESEARCH INSTITUTES

### TELETHON INSTITUTE OF GENETICS AND MEDICINE (TIGEM)

The Telethon Institute of Genetics and Medicine (TIGEM), founded in 1994, focuses its current activity on disease gene identification, functional studies (both in vitro and in vivo) and gene therapy of eye disorders.



Early work at TIGEM was centered on disease gene identification and mutation analysis and contributed to making it an international reference in those fields. Later on, TIGEM researchers moved to functional genomics to address the pathogenetic mechanisms of a number of hereditary disorders; more recently, they have addressed functional studies and gene therapy approaches in animal models of genetic diseases. TIGEM researchers have recently contributed to a successful clinical trial for the gene therapy of Leber congenital amaurosis, an inherited retinal disorder (see also the

“Achievements of Telethon Research” section).

TIGEM’s laboratories, located in Naples, are part of a developing science and technology research area, which also includes the CNR's International Institute of Genetic and Biophysics (IIGB) and Institute of Protein Biochemistry (IBP). [Andrea Ballabio](#), an internationally reputed geneticist, has been the Scientific Director of TIGEM since its foundation.

TIGEM is totally owned and operated by Telethon and its funding is reviewed every five years, the next review being in 2011. TIGEM has a current staff of 96, including 12 group leaders, 70 junior investigators, and technicians and 14 administrative and support staff. Details on TIGEM are available on the Institute's website [www.tigem.it](http://www.tigem.it)

### HSR-TELETHON INSTITUTE OF GENE THERAPY (HSR-TIGET)

The HSR- Telethon Institute of Gene Therapy (HSR-TIGET), founded in 1995, is located in Milan and is devoted to the development of gene therapy protocols for inherited diseases.



Scientists at the HSR-TIGET are international leaders in the field of gene therapy for hereditary diseases of the immune and hematopoietic system and for genetic neurodegenerative and neuromuscular disorders, pursuing basic and applied research on these areas.

In recent years, the institute has invested significantly in the use of lentiviral vectors as promising new tools for targeting gene transfer to hematopoietic stem cells. Dedicated facilities for the diagnosis, treatment and follow up of patients enrolled in gene therapy clinical trials are provided by an in-house Pediatric Clinical Research Unit established in 2000.

Researchers at the HSR-TIGET have made major breakthroughs both in basic research and are at the forefront of clinical applications. A major achievement was the first successful and safe gene therapy

treatment for ADA-SCID, a severe inherited immunodeficiency, which is today the golden standard for the cure of this disorder (see also the “Achievements of Telethon Research” section further in this document).

HSR-TIGET is a joint initiative between the San Raffaele Foundation and Fondazione Telethon, and splits the costs equally: Telethon contributes essentially to the direct expenses of the Institute (i.e. soft money), whereas the San Raffaele Foundation covers fixed costs, providing equipped space and contributing to the cost of personnel with tenure track positions. HSR-TIGET’s funding is reviewed every five years, the next review being in 2011.

[Luigi Naldini](#), an internationally-recognized leader in lentiviral gene therapy has been HSR-TIGET’s Scientific Director since 2008.

HSR-TIGET has a staff of 98, including 14 group-leaders/project leaders, 79 junior researchers and technicians and 5 administrative and support staff.

Details on HSR-TIGET are available [on the Institute’s website](#).

## DULBECCO TELETHON INSTITUTE (DTI)

The Dulbecco Telethon Institute (DTI) is a “disseminated institute” whose principal investigators are named “*Dulbecco Telethon Scientists*”. The DTI is not housed in a single campus; instead, DTI Scientists conduct their work at various leading research institutions nationwide. The DTI represents a new enterprise in the Italian scientific landscape: working on a broad range of topics and in different Italian Institutions, these talented scientists share the same principles of excellence in pursuing scientific research aimed at understanding, preventing and curing genetic diseases.

The program is highly competitive and provides funds for five years to gifted independent scientists to establish their own groups in Italian host Institutes. Three position levels are available; by the end of the first 5-year term, Telethon Scientists can apply for a promotion to the next career level, which is evaluated by Telethon Scientific Committee.

There are currently 25 working DTI principal investigators, including one Senior, 13 Associate and 11 Assistant Telethon Scientists, respectively. The DTI has a total staff of 122, including technicians and fellows. Details on all DTI laboratories are available on the [DTI website](#).



## EXTRAMURAL RESEARCH

### RESEARCH PROJECTS

Once a year, Telethon issues a call for applications for extramural research grants. Applications may include a single project under the responsibility of one principal investigator, or multicentric projects, led by a program coordinator, involving a team of partner investigators. A joint Telethon-UIDLM call for clinical project is also issued annually (see the box on “Fostering Research on Neuromuscular Diseases” below).

The success rate of submitted applications was 13% (40 projects funded) in the 2009 calls, a rate consistent with the stringent criteria of selection adopted for the Telethon review process.

### RESEARCH SERVICES AND CORE FACILITIES

Telethon’s annual call for application for research services and core facilities aims at offering complex technologies and variety of tools and biological samples to researchers, such as transgenic, imaging/microscopy, proteomics and bioinformatic facilities. Telethon service and facilities are funded on a fee-for-service basis, and Telethon covers 50% of the costs for use by Telethon-funded laboratories.

More information on Telethon research services and facilities is available on [Telethon’s website](#).

### GENETIC BIOBANKS NETWORK

Within the call for research service and core facilities, Telethon invites annual applications to fund new biobanks (banks of biological samples) whose aim is to collect, preserve and offer to the scientific community biological samples and related clinical data from individuals affected by genetic diseases, their relatives and healthy control individuals. To optimize such initiative, a network of all Telethon biobanks was established to coordinate and manage their activities, providing scientists with an efficient service and high, consistent quality standards. Among other things, the network ensures the same quality criteria, a common IT interface and standard operating procedures. Operative and ethical guidelines are provided by a common charter with which all biobanks comply.

The network currently includes eight biobanks, with a catalogue of about 40,000 samples, a significant collection in the field of genetic diseases, many of which are rare.

Details of the genetic biobank network are available on the [network website](#).

### PROGRAM PROJECTS

As specified in the Strategic Plan for Research, in 2009 Telethon issued its first call for application for Program Project grants. The aim of this program is to award collaborations between scientists involved in clinical, pre-clinical and basic research to allow innovative translational approaches and progress towards a cure for genetic diseases. Synergy and a multidisciplinary approach are two distinctive features of Telethon program projects: applications must include a minimum of three to a maximum of six projects from different investigators, of which at least one in clinical or late-stage pre-clinical phase.

Projects are required to share a set of clearly defined goals around a central theme that is highly relevant to Telethon’s mission of moving towards the cure of genetic diseases. As a general criterion, the impact of the Program Project as a whole should be greater than the sum of those of the single projects taken separately. Each call will award one Program Project with a maximum budget of 1,800,000 € for 3 years. The first call for letters of intent and application, issued in May 2009, is currently under review.

### FOSTERING RESEARCH ON NEUROMUSCULAR DISEASES

Neuromuscular disorders, a large group of disorders affecting muscular function, were the early focus of Telethon activity and are still of paramount interest for its research. Besides funding a number of projects devoted to neuromuscular diseases through its regular grants, Telethon is currently pursuing a range of initiatives to specifically foster research on this group of disorders:

☒ Setting up the clinical infrastructure: TREAT NMD

Telethon is among the partners of Treat NMD , a networking initiative funded within the European Sixth Framework Program, with the aim of advancing diagnosis and care for people with neuromuscular diseases and creating the infrastructure to ensure that the most promising new therapies reach patients as quickly as possible (<http://www.treat-nmd.eu/home.php>).

☒ Telethon-UILDM clinical grants

Since 2002, this joint grant program initiative with the Italian Muscular Dystrophy Association UILDM distributes funds to clinical research projects aimed at improving the quality of life of patients affected by genetic muscle and nerve disorders. An educational program in collaboration with biostatisticians is offered to applicants to provide methodological support for the definition of the clinical protocol and study design.

☒ Neuromuscular Biobanks

The EuroBioBank network, coordinated by the European Organisation for Rare Diseases, is the first network of biobanks in Europe providing human DNA, cell and tissue samples to the scientific community conducting research on rare diseases. Telethon is currently funding 5 out of the 13 biobanks in the network, accounting for the entire Italian branch of the project(<http://www.eurobiobank.org>).

☒ Funding research on ALS

Telethon is a founding member of ARISLA, a private, non-profit funding agency devoted to fostering research towards the cure of Amyotrophic Lateral Sclerosis, a severe neuromuscular disorder (<http://www.arisla.org/>). Started at the end of 2008, AriSLA launched its first Call for Ideas in 2009.

☒ The European NeuroMuscular Centre (ENMC)

Telethon is a member of ENMC, a consortium of international neuromuscular patient organizations, whose main scope is to facilitate communication among scientists, clinicians and persons affected by neuromuscular diseases. The rotating Chair of the ENMC Executive Committee is held by Telethon for 2009-2011 (<http://www.enmc.org/>).

# THE PEER-REVIEW PROCESS AT TELETHON

## OVERVIEW

All Telethon funding decisions are subordinate to a peer review process. The Scientific Office manages the development and implementation of policies and procedures that pertain to peer review, while leaving complete control over funding decisions to the Scientific Committee.

A staff of professionals with a scientific background manages the peer-review of projects and is responsible for overseeing and implementing the strategic plan for Telethon research, ensuring that every aspect of research is consistent with Telethon's mission and priorities.

## CRITERIA OF EVALUATION

The evaluation of projects is based on a scoring system that takes into account three criteria:

- a) scientific merit;
- b) relevance to Telethon's mission;
- c) proximity to cure (score is higher for projects that are closer to a clinical application)

The relative weight of the scientific merit being predominant in the overall score, scientific excellence remains the main criterion for Telethon funding.

## PHASES OF THE PEER-REVIEW PROCESS AT TELETHON

Applications, which are submitted electronically, are initially screened for administrative compliance by the Telethon staff. The procedure includes external and internal reviews; applications are sent to a minimum of two ad-hoc external reviewers chosen by the Scientific Office among experts in the field, as well as to three members of the Scientific Committee (internal reviewers). An interview is included in the selection of candidates for the Dulbecco Telethon Institute.

Final decisions are taken during a study section of the Scientific Committee. Written feedback, including the reviewers' comments and a summary of the plenary discussion, is provided to applicants.

## THE TELETHON SCIENTIFIC COMMITTEE

The Telethon Scientific Committee is composed of scientists of international standing, serving a four-year term. To minimize potential conflicts of interest, Italian members working in Italy are currently less than 10%.

Members of the Scientific Committee are asked: a) to review applications for external research grants arising from competitive calls; b) to be involved in the revision of Telethon's Institutes TIGEM and HSR-TIGET and in their periodic assessments through site visits; c) to review applications and promotions for the position of Telethon Scientist at the Dulbecco Telethon Institute (DTI).

The current list of members of the Telethon Scientific Committee is available online on the [Telethon's website](#).

## ACHIEVEMENTS OF TELETHON RESEARCH

In the long term, Telethon's success is measured by the fact that its research leads to new discoveries and that these discoveries will lead to clinical applications. Telethon's clinical pipeline, described below, includes project ranging from the late pre-clinical phase to successful clinical trials for various hereditary disorders. The path from discovery to clinical application, however, is a long and incremental process: therefore, to regularly assess our progress, we also make use of bibliometric indicators that measure the quality of Telethon funded publications and the scientific progress it produces.

### TELETHON CLINICAL PIPELINE

#### Internal research

Among the gene therapy projects carried out in Telethon's institutes HSR-TIGET and TIGEM, five are currently at a clinical or advanced preclinical stage of development (figure 4).

A gene therapy protocol for **ADA-SCID**, an inherited immunodeficiency caused by a defect in the gene coding for adenosine deaminase (ADA), was pioneered by scientist at the HSR-TIGET, who have so far successfully treated 13 children so far, all of which have completely recovered from the immunodeficiency and are now conducting a normal life. The HSR-TIGET protocol was recognized as the first successful and safe gene therapy trial for a genetic disease and it is now the international golden standard for the therapy of ADA-SCID. The therapy has obtained Orphan Drug designation from the European Medicine Agency EMEA and the FDA in 2005 and 2009, respectively.

Gene therapy for **Wiskott-Aldrich Syndrome** (WAS), a rare genetic immunodeficiency, and **Metachromatic leukodystrophy** (MLD), a severe lysosomal storage disorder, involving lentiviral vectors developed by scientists at HSR-TIGET, have currently completed the preclinical phases; clinical trials in patients are due to start within the first half of 2010.

Gene therapy for **Beta-thalassemia**, an inherited blood disease, and for **Stargardt disease**, a genetic disorder of the retina, are in late and early preclinical phases at the HSR-TIGET and TIGEM, respectively.

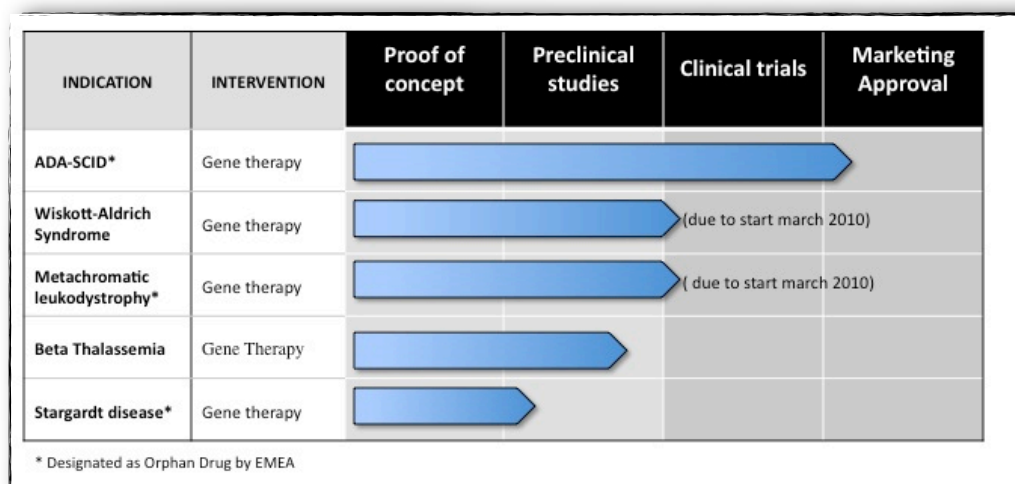


Figure 4: clinical pipeline of Telethon research Institutes. *Source: Telethon.*

### Telethon external grants and international collaborations

Results arising from Telethon external grants and international collaborations are providing significant contributions towards the development of therapies for a number of genetic diseases, some of which have already reached clinical or advanced preclinical phases (figure 5).

TIGEM researchers have played a part in a successful pilot clinical trial for the gene therapy of **Leber congenital amaurosis**, an inherited retinal disorder leading to blindness, carried out at the Children's Hospital in Philadelphia, which is now moving into later clinical phases.

Telethon grants are supporting the follow-up a pharmacological pilot study which has given promising results for the treatment of **Bethlem/Ullrich muscular dystrophy**; the planning of a stem cell therapy trial for **Duchenne muscular dystrophy**, and a 'proof of concept' study to test the effectiveness of chaperones as an enhancement therapy in addition to the currently available enzyme replacement protocol in patients with Pompe disease.

Moreover, a number of projects are providing significant proof of concepts for the therapy of various diseases in animal models and in vitro; many of them are approaching to the preclinical phases.

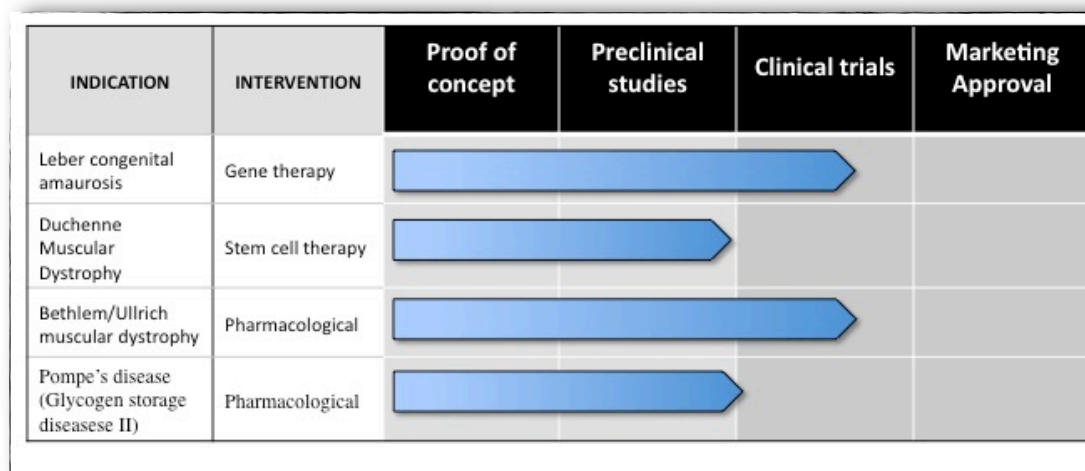


Figure 5: clinical pipeline of Telethon external projects and international collaborations. *Source: Telethon*



## BIBLIOMETRIC INDICATORS

Statistics on the number and quality of peer-reviewed publications (referred to as bibliometric indicators) are standard tools used to monitor and benchmark scientific productivity and are therefore a useful indicator to assess the quality of Telethon funded-research and the scientific progress it makes.

The average citation index of Telethon-funded publications (figure 6) has constantly increased since 1991 and is now higher than the Italian, European and US average in five relevant areas of biomedicine. The average citation index measures the average number of times that a publication has been cited in the literature, a commonly used indicator of its quality.

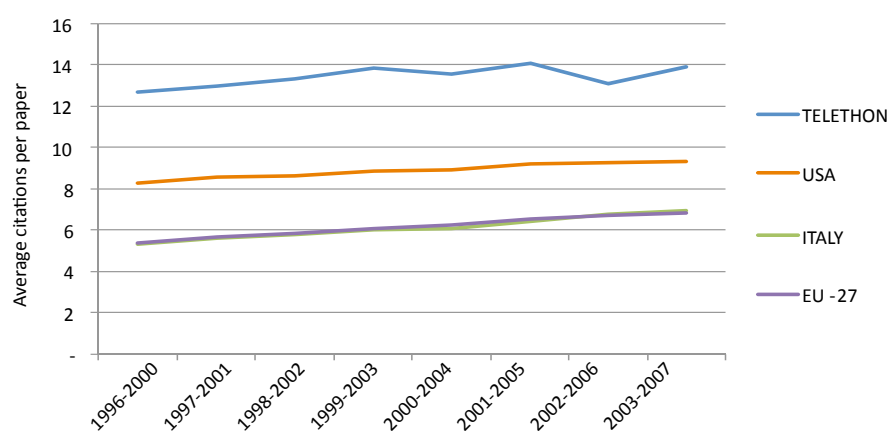


Figure 6. Average citations of publications arising from Telethon-funded research, compared to the Italian, USA and European averages in five relevant areas of biomedicine (values shown are the mean of five areas: Biology and Biochemistry, Clinical Medicine, Immunology, Molecular Biology and Genetics, Neuroscience and Behaviour). *Source: Thomson Reuters (ISI), Philadelphia.*

## PATENTS AND TECHNOLOGY TRANSFER

The **Telethon Technology Transfer Office (TTTO)**, was created in 2005 to protect the intellectual property of Telethon results and to promote their transfer to the industry.

The main goal is to allow the development and commercialization of drugs, therapies and diagnostics for the treatment of muscular dystrophy and other genetic diseases, making them available to patients. The current strategy involves the pursuit of partnerships with pharmaceutical companies that may provide financial support, resources and expertise for the development and exploitation of Telethon's clinical programs. Given the extreme rarity of the diseases being addressed, establishing these partnerships is seen as a necessary though ambitious goal. The TTTO is currently focusing its activity on patentable inventions made by scientists working in its three Institutes (TIGEM, HSR-TIGET, DTI).

To date, Telethon has patented 22 inventions, of which 5 are currently licensed to third parties.

Details on the TTTO activities and information on licensing opportunities can be found at:

<http://www.telethon.it/sites/researchers/technologytransfer/default.aspx>



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<http://www.telethon.it/sites/researchers/strategicplan/default.aspx>