FONDAZIONE Telethon

Social Report **2024**: Fondazione Telethon in numbers

WHO WE ARE

Fondazione Telethon is an Italian non-profit organization committed to advancing research on rare and complex genetic diseases. For over 35 years, we have supported high-impact science aimed at developing innovative treatments and improving the lives of people affected by these conditions.

OUR MISSION

1. To develop innovative treatments and therapies, aiming to translate research into real-world health benefits.
2. To fund cutting-edge research on genetic diseases, with a focus on rare and underserved conditions.
3. To help shape regulatory frameworks that ensure high standards and sustainability in the development of gene and cell therapies.
4. To raise awareness about the burden of genetic diseases and the importance of sustained scientific and societal commitment.

OUR ACHIEVEMENTS AND CAPABILITIES

- Two intramural research institutes (Tigem and SR-Tiget) employing over 500 scientists with deep expertise in gene therapy, molecular biology, and translational medicine.
- Pioneering work in gene therapy, including contributions to the development and approval of Strimvelis and Libmeldy, with a third therapy (for Wiskott-Aldrich Syndrome) expected to be approved by both FDA and EMA in late 2025.
- Extensive clinical expertise in a range of conditions, including primary immunodeficiencies, neuromuscular diseases, metabolic and lysosomal storage disorders, blood diseases, and inherited retinal disorders.
- Strategic partnerships with leading hospitals and academic institutions such as Ospedale “San Raffaele” (Milan), Ospedale Universitario “Federico II” (Naples), and Università degli Studi della Campania “Luigi Vanvitelli” (Caserta).
- Tech transfer leadership: Fondazione Telethon is a leader in technology transfer, actively fostering collaborations with the pharmaceutical and biotech industries to maximize the value of scientific discoveries and strengthen Italy’s research ecosystem.

Administrative management: revenues and expenses 2024

/ TOTAL REVENUES 2024 AND COMPARISON WITH 2023 (THOUSANDS OF EUROS)

REVENUES	2024	2023
Revenues from institutional activities	22,349	22,302
Revenues from fundraising (*)	72,808	62,274
Financial and asset revenues	1,822	1,926
TOTAL REVENUES	**96,979**	**86,502**

(*) Includes values recorded in the financial statements under fundraising, 5x1000 contributions, and other donations attributable to fundraising activities.

/ TOTAL EXPENSES 2024 AND COMPARISON WITH 2023 (THOUSANDS OF EUROS)

EXPENSES	2024	2023
Mission-related expenditures	62,622	58,343
- *Scientific research*	54,153	52,024
- *Research support activities*	8,469	6,319
Fundraising expenses	27,210	23,330
General support expenses	3,088	3,327
Financial and asset expenses	1,470	336
TOTAL EXPENSES*	**94,390**	**85,336**

(*) includes taxes

/ FOCUS ON EXPENDITURES AND COSTS: WHERE OUR FUNDS WENT

INVESTMENTS IN SCIENTIFIC RESEARCH

€ 54,153 million

- TIGEM (Telethon Institute of Genetics and Medicine)
- SR-TIGET (San Raffaele Telethon Institute for Gene Therapy)
- Competitive and ad hoc research grants
- Research support
- Patient support (e.g., Come a Casa)

INVESTMENTS IN COMMUNICATION AND RESEARCH MANAGEMENT

€ 8,469 million

- Research management
- Networked associations
- Info_rare service
- Awareness activities on genetic diseases and their impact on patients' lives
- Educational projects

GENERAL SUPPORT EXPENSES

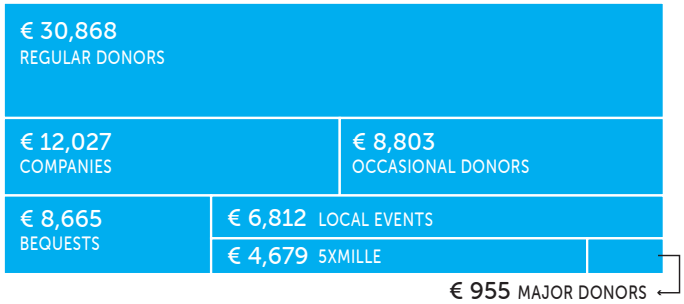
€ 3,088 million

FUNDRAISING EXPENSES

€ 27,210 million

/ FUNDRAISING EFFICIENCY: HOW MUCH IT COST TO RAISE 1 EURO

/ SOURCES OF FUNDS RAISED (THOUSANDS OF EUROS)



/ PEOPLE WORKING FOR FONDAZIONE TELETHON AS OF 12/31/2024

EMPLOYEES

OF WHICH: WOMEN / MEN

COLLABORATORS

OF WHICH: WOMEN / MEN

2024 numbers from our institutes

/ TELETHON INSTITUTE OF GENETICS AND MEDICINE (POZZUOLI – NAPLES)

15.2 MILLION
INVESTED BY FONDAZIONE TELETHON. SINCE 1994, THE FOUNDATION HAS INVESTED A TOTAL OF €147 MILLION IN THE INSTITUTE'S ACTIVITIES

2
CLINICAL STUDIES IN PROGRESS (MUCOPOLYSACCHARIDOSIS TYPE 6 AND USHER SYNDROME TYPE 1B)

1
NATURAL HISTORY STUDY ON USHER SYNDROME TYPE 1B

/ INSTITUTE STAFF AS OF 12/31/2024

1
ERC GRANT IN 2024

/ TIGEM'S STRATEGIC RESEARCH AREAS

CELL BIOLOGY | GENOMIC MEDICINE | MOLECULAR THERAPY

2024 numbers from our Institutes

/ SAN RAFFAELE TELETHON INSTITUTE FOR GENE THERAPY (MILAN)

15.6 MILLION
INVESTED BY FONDAZIONE TELETHON. SINCE 1996, THE FOUNDATION HAS INVESTED A TOTAL OF €161 MILLION IN THE INSTITUTE'S ACTIVITIES

2 THERAPIES
REGISTERED AS MEDICINES FOR ADA-SCID AND METACHROMATIC LEUKODYSTROPHY

1 DISEASE
FOR WHICH A REGISTRATION REQUEST HAS BEEN SUBMITTED BOTH IN EUROPE AND THE USA (WISKOTT-ALDRICH SYNDROME)

/ INSTITUTE STAFF AS OF 12/31/2024

TOTAL STAFF	276	
OF WHICH WOMEN / MEN	205	71
PROJECT LEADERS	22	

3
ERC GRANTS IN 2024

/ SR-TIGET'S STRATEGIC RESEARCH AREAS

- EX VIVO GENE THERAPY OF HEMATOPOIETIC STEM CELLS FOR RARE GENETIC DISEASES
- IN VIVO GENE THERAPY USING LENTIVIRAL VECTORS
- GENETIC AND EPIGENETIC EDITING
- GENE AND CELL THERAPY STRATEGIES FOR MODULATING THE IMMUNE RESPONSE
- GENE THERAPY STRATEGIES FOR CANCER IMMUNOTHERAPY

Fondazione Telethon's extramural research in 2024

/ MULTIROUND CALL: STUDY OF DISEASE MECHANISMS AND IDENTIFICATION OF THERAPEUTIC APPROACHES

€10.3M
INVESTED BY THE FOUNDATION

58
PROJECTS FUNDED

63
RARE GENETIC DISEASES STUDIED

/ FONDAZIONE TELETHON - FONDAZIONE CARIPLO JOINT CALL: BASIC RESEARCH AND FOCUS ON GENE FAMILIES

€1.6M
INVESTED BY THE FOUNDATION

14
PROJECTS FUNDED FOR 15 DISEASES

22
RESEARCH GROUPS INVOLVED

15
RARE GENETIC DISEASES STUDIED

/ SEED GRANT: A CALL IN COLLABORATION WITH PATIENT ASSOCIATIONS TO SUPPORT EXPLORATORY STUDIES ON RARE GENETIC DISEASES

€724 thousand
INVESTED, INCLUDING:

→ **604** BY PATIENT ASSOCIATIONS
120 BY FONDAZIONE TELETHON

14
PROJECTS FUNDED, INCLUDING:

→ **12** BY PATIENT ASSOCIATIONS
2 BY FONDAZIONE TELETHON

/ CLINICAL GRANT FROM FONDAZIONE TELETHON WITH UILDM: CLINICAL PROJECTS AIMED AT IMPROVING THE QUALITY OF LIFE FOR INDIVIDUALS

€1.53M
INVESTED BY THE FOUNDATION

6
RESEARCH PROJECTS

55
CLINICAL RESEARCHERS

32
RESEARCH INSTITUTIONS

/ PLATFORM FOR DUCHENNE MUSCULAR DYSTROPHY

16 centers involved in a special project for the development of a digital platform dedicated to the natural history of Duchenne muscular dystrophy.

/ PLATFORM FOR DUCHENNE MUSCULAR DYSTROPHY: STUDY OF DISEASE MECHANISMS AND IDENTIFICATION OF THERAPEUTIC APPROACHES

€2.8M
INVESTED BY THE FOUNDATION

8
RESEARCH GROUPS FUNDED

Target diseases

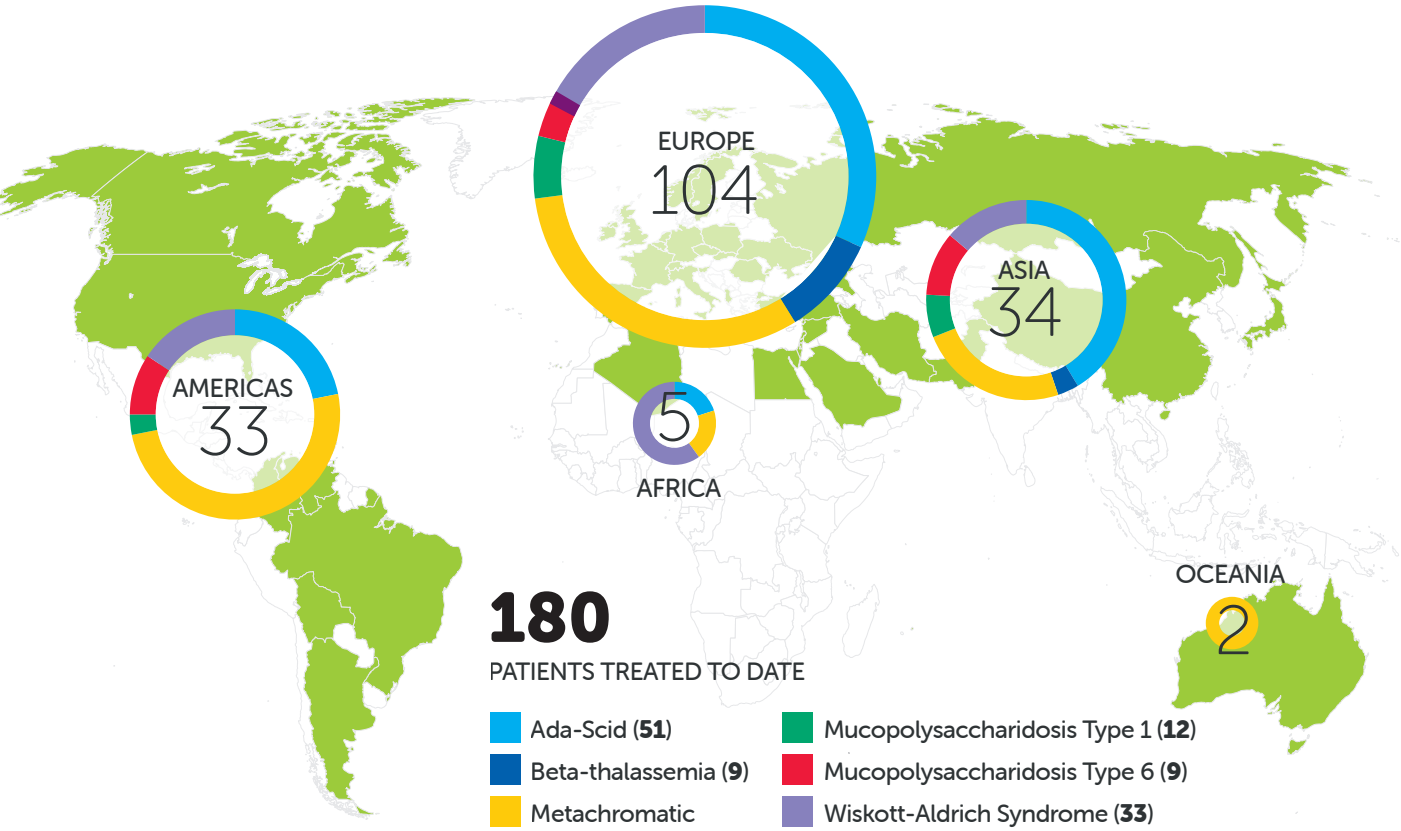
> DUCHENNE MUSCULAR DYSTROPHY
> SPINAL AND BULBAR MUSCULAR ATROPHY
> (KENNEDY'S DISEASE)
> MITOCHONDRIAL MYOPATHY (POLG GENE)

Fondazione Telethon's therapies (12/31/2024)

Whenever possible, Fondazione Telethon collaborates with pharmaceutical companies to develop and make available therapies for rare genetic diseases. However, this path is not always feasible. For this reason, in 2023, it launched an alternative model of production and distribution, currently unique at the international level. The goal is not to replace the industry, but to ensure access to existing therapies and make sure that new treatments truly reach those who need them.

The Foundation's impact on people

/ FROM ALL OVER THE WORLD FOR TREATMENT WITH GENE THERAPIES DEVELOPED AT FONDAZIONE TELETHON INSTITUTES (07/31/2025)



180
PATIENTS TREATED TO DATE

- Ada-Scid (**51**)
- Beta-thalassemia (**9**)
- Metachromatic leukodystrophy (**57**)
- Mucopolysaccharidosis Type 1 (**12**)
- Mucopolysaccharidosis Type 6 (**9**)
- Wiskott-Aldrich Syndrome (**33**)
- Usher Syndrome Type 1B (**9**)

/ COME A CASA (12/31/2024)

A program supporting that supports families and patients throughout the treatment journey.

120 PATIENTS WELCOMED SINCE 2016

13 IN 2024

/ UNDIAGNOSED DISEASES PROGRAM

93
CASES ANALYZED IN 2024

27
CASES DIAGNOSED IN 2024

6
SCIENTIFIC PUBLICATIONS IN 2024

/ Info_Rare

A free service for those seeking information on rare genetic diseases.

1,816
REQUESTS HANDLED IN 2024

Fondazione Telethon's numbers from 1991 to 2024

741

MILLIONS OF EUROS INVESTED IN RESEARCH, INCLUDING OVER €43 MILLION IN 2024

1,916

RESEARCHERS FUNDED, INCLUDING 41 FUNDED BY FONDAZIONE TELETHON FOR THE FIRST TIME IN 2024

661

DISEASES STUDIED, INCLUDING 24 FUNDED BY FONDAZIONE TELETHON FOR THE FIRST TIME IN 2024

255

RESEARCH INSTITUTIONS FUNDED, INCLUDING 3 FUNDED IN 2024 BY FONDAZIONE TELETHON FOR THE FIRST TIME

3,118

PROJECTS FUNDED, INCLUDING 94 LAUNCHED IN 2024

15,366

TOTAL PUBLICATIONS, INCLUDING 370 IN 2024

91

INVENTIONS PATENTED BY TELETHON RESEARCHERS AND STILL ACTIVE, INCLUDING 9 FILED IN 2024

20

ACTIVE ORPHAN DRUG DESIGNATIONS BY FONDAZIONE TELETHON AT THE END OF 2024

239

CALLS FOR FUNDING EXTRAMURAL RESEARCH, INCLUDING 16 IN 2024

1

DISEASE FOR WHICH A REGISTRATION REQUEST HAS BEEN SUBMITTED BOTH IN EUROPE AND IN THE USA (WISKOTT–ALDRICH SYNDROME)

2

THERAPIES REGISTERED AS MEDICINES FOR ADA-SCID AND METACHROMATIC LEUKODYSTROPHY

From Bench to Bedside

Fondazione Telethon has become a **leading example in Italy** for translating scientific discoveries into approved therapies that reach patients and remain available over time. Through **licensing agreements and strategic collaborations with the pharmaceutical industry**, we have successfully brought gene therapies developed in our laboratories to the market.

- Thanks to advanced research by Fondazione Telethon and collaboration with the pharmaceutical industry, the world's first stem cell gene therapy for ADA-SCID, a severe congenital immunodeficiency, became available. In 2023, Fondazione Telethon **assumed full responsibility for its marketing authorization, production, and distribution in Europe**, ensuring continued access for eligible patients.
- Fondazione Telethon research also led to a gene therapy for **metachromatic leukodystrophy (MLD)**, a severe neurodegenerative disease. **Approved by the EMA in 2020 and the FDA in 2024**, the therapy resulted from nearly **two decades of work at Fondazione Telethon's SR-Tiget Institute in Milan, in collaboration with Orchard Therapeutics**.
- Gene therapy for another immunodeficiency, **Wiskott-Aldrich syndrome**, may soon follow. For this condition, in addition to research, **Fondazione Telethon is pursuing the regulatory approval process with both the FDA and EMA**.

Future Directions and Collaboration Opportunities

To amplify our impact, Fondazione Telethon is open to strategic collaborations that:

→ **ACCELERATE ACCESS TO GENE THERAPIES** AND CLINICAL TRIALS FOR RARE DISEASES

→ **ESTABLISH NEW RESEARCH PROGRAMS** IN AREAS OF UNMET NEED

→ **EXPAND INTERNATIONAL NETWORKS** AND SHARE SCIENTIFIC EXPERTISE AND INFRASTRUCTURE

→ **EXPLORE FUNDING PARTNERSHIPS** TO SUSTAIN LONG-TERM RESEARCH AND DEVELOPMENT

> *Every day Fondazione Telethon proves that, where industrial logic fails due to insufficient return on investment, it is possible to apply a different and virtuous model that leaves no one behind and aims to become industrially sustainable over time.*
>
> Ilaria Villa, *General Director, Fondazione Telethon*

FONDAZIONE Telethon

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