

About the Future: from Knowledge Transfer to Impact

Fondazione Telethon's
perspective

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**EVERY MINUTE OF EVERY DAY,
TEN CHILDREN AROUND THE WORLD
ARE BORN WITH A RARE GENETIC DISEASE.
TOMMASO IS ONE OF THEM.**

Who we are: Mission & Vision



- **Fondazione Telethon** is a **major Italian biomedical charity** focused on **genetic diseases**
- **Founded in 1990** at the behest of a group of **patients**
- Supported through **fundraising**

OUR MISSION

Advance biomedical research towards the cure of genetic diseases

528 M€ research investment*

2,632 research grants

>11.000 papers published

1,612 PIs awarded

571 genetic diseases studied

OUR VISION

Convert the results of excellent, selected and sustained research into available therapies

12 active clinical trials

101 patients treated

1 Therapy on the market

* 2018 Balance sheet



Telethon supports the Italian research system through competitive calls



EXTRAMURAL RESEARCH: Projects selected through competitive calls for grants

✓ FLEXIBILITY
✓ OPPORTUNITIES

TELETHON'S ROLE:
no IP ownership
funder and catalyst

- **Peer review process** by an **independent** scientific committee and several external reviewers ensuring that grants are awarded to the best projects
- ISO9001:2015 **quality certified peer review process**
- A variety of calls from exploratory projects to multicentre translational projects
- Call for clinical research projects in the **neuromuscular** field
- Yearly call for proposals attracting **hundreds of applicants**
- **Broad coverage** of diseases and approaches
- Foreseen overall commitment around 11 million euros in 2019

The peer review process: max 3y competitive fund allocation

Evaluation of projects by independent reviewers, on the basis of the American NIH model. The process is managed by internal Research Program Managers



Telethon supports its own institutes in Italy



INTRAMURAL RESEARCH: Three Telethon institutes financed by Telethon

✓ FOCUS
✓ CRITICAL MASS

TELETHON'S ROLE:
IP owner
Developer



Telethon Institute of Genetics and Medicine

Location: Pozzuoli, Naples

Director: Andrea Ballabio

Group leaders: 16

Staff: 218



Telethon Institute for Gene Therapy

Collaborative venture with: San Raffaele Hospital

Location: Milan

Director: Luigi Naldini

Group leaders: 12

Staff: 181



Dulbecco Telethon Institute (virtual Institute)

Current locations: Trento, Milan, Padua, Naples, Genoa, Monza

Group Leaders: 11

The peer review process: 5y non competitive fund allocation



Grant Renewal
Proposals



Program
Proposals
Submission



Scientific
Evaluation



Site Visit



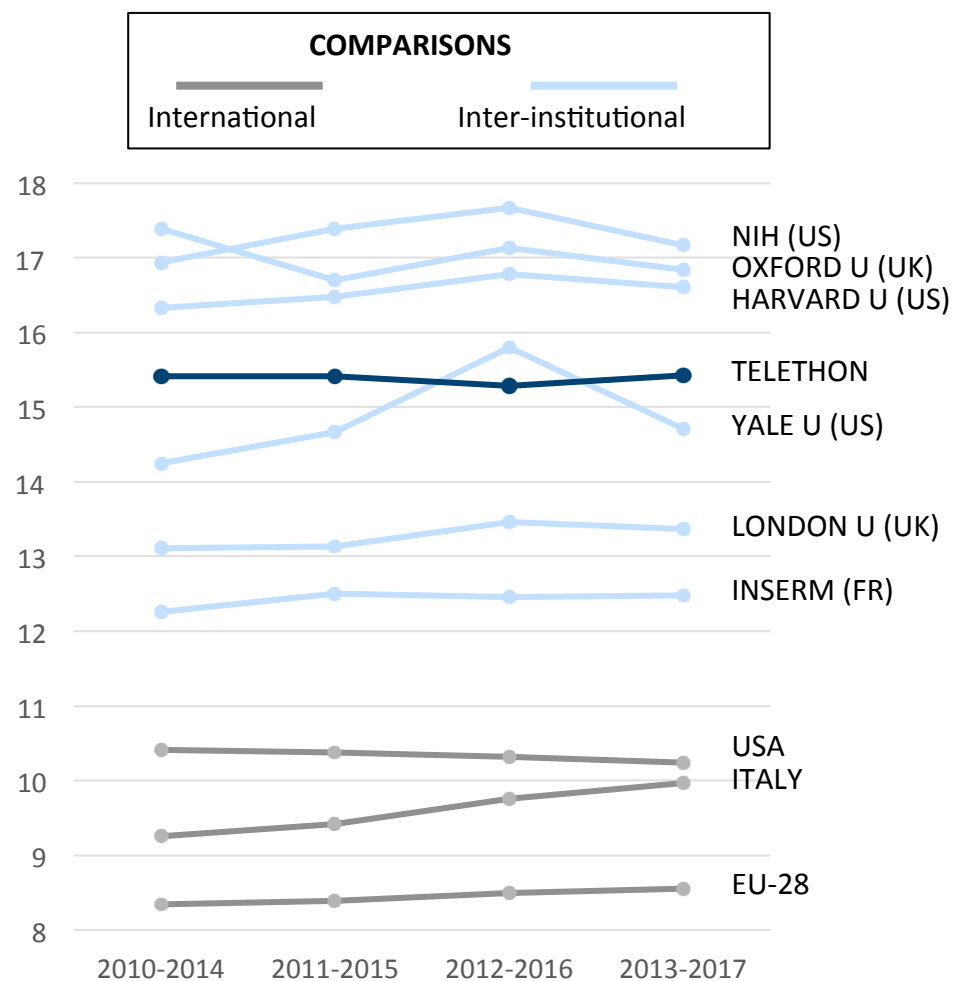
Feedback to
researchers

Telethon's publications keep comparing favorably in international comparisons by different indicators



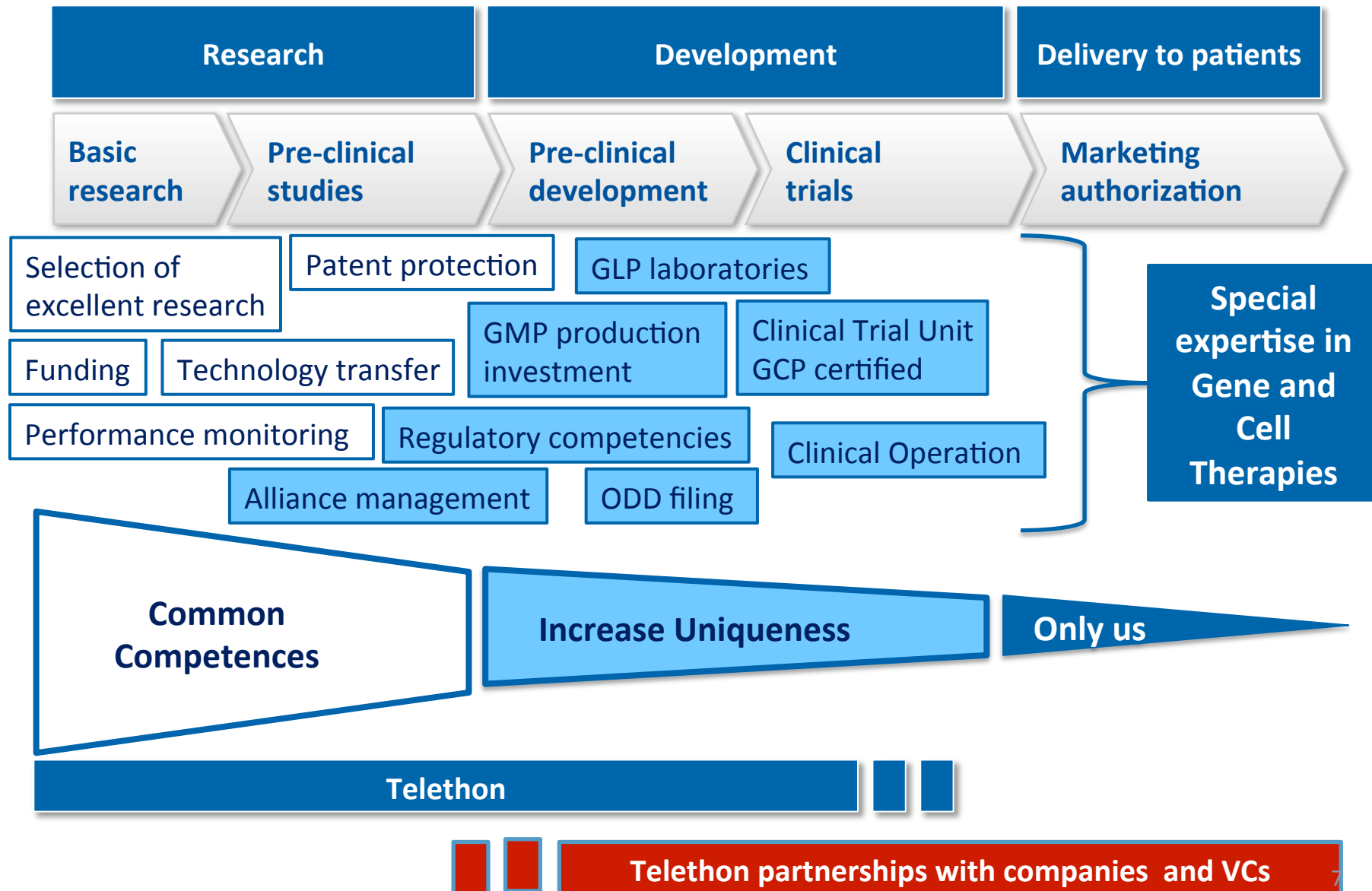
Average number of citations/paper

Original articles and reviews in the five major life sciences categories
(95% of all Telethon publications)



Sources: Clarivate Analytics (left panel); November 2018

We have unique expertise: Telethon's translational competences



We team with industrial partners: Our Major Alliances



	Start year	Institute	Scope
	2018		Exclusive license on retroviral-based ex vivo gene therapy for ADA-SCID , and lentivirus based for WAS, MLD, beta thalassemia, MPS1 .
	2011		Small molecule drug candidates for Lysosomal Storage disorders and neurodegenerative diseases
	2012		Gene therapy and small molecule approach for the treatment of Lysosomal Storage disorders and neurodegenerative diseases
	2018		Lentivirus-based gene therapy for blood disorders
	2016		Genome editing of hematopoietic stem cell (HSC) and T cell therapies
	2016		Undisclosed
	2018		lysosomal storage disorders and more common disorders such as neurodegeneration .

The pillars of Telethon's industrial agreements



All agreements between Telethon and industrial Partners

- Safeguard **research independence** of Telethon investigators
- Retain **intellectual property rights**
- Mandate **commitment in developing therapies**
- Imply **return of any IP** and **results co-developed**, in case the Partner does not pursue therapy development
- Provide **funding** in support of the research in the collaboration programs
- Supply **additional funding** through milestones/royalties, in support of further research activities

ITAtech initiative



- **ITAtech** is the first joint initiative between Cassa depositi e prestiti (CDP) and the European Investment Fund (EIF) supporting «Technology Transfer» through a 200M€ capital fund
- **Aims:**
 1. Accelerate and support technology transfer of Italian Research results
 2. Promote and boost the creation of technology transfer professionals with a strong expertise on selected technological sectors
- **Key stakeholders:** public and private entities active in technology transfer as universities , research centers, tech transfer offices, start ups, early stage investors
- **Investment target:** highly innovative and technological projects

SOFINNOVA

PARTNERS FOR LIFE



45 YEARS OF VENTURE CAPITAL



€2B DEDICATED TO LIFE SCIENCES



EUROPE FOCUSED – GLOBAL REACH



**FUNDING ENTREPRENEURS OF
GAME-CHANGING COMPANIES**

Key Sofinnova Telethon Fund terms



SOFINNOVA
PARTNERS FOR LIFE

- **Up to €100M (€93M already raised)** fund managed by Sofinnova Partners
- Dedicated to **Italy**, focused on **rare/genetic** diseases
- **Strategic partnership** with Fondazione Telethon
- Experienced **dedicated team** with complementary skills
- **15-20** investments: 10-15 seeds, 4-5 series A
- Maximum investment size **€10M**
- **Investing...**

The Sofinnova Telethon Fund: maximize complementarity



SOFINNOVA
PARTNERS FOR LIFE

FONDAZIONE



SOFINNOVA
PARTNERS FOR LIFE

IDENTIFY

- ▶ Capillary knowledge of the Italian research system
- ▶ Access to the best in class researchers nationwide
- ▶ Capability to guide institutes and researchers toward technology transfer

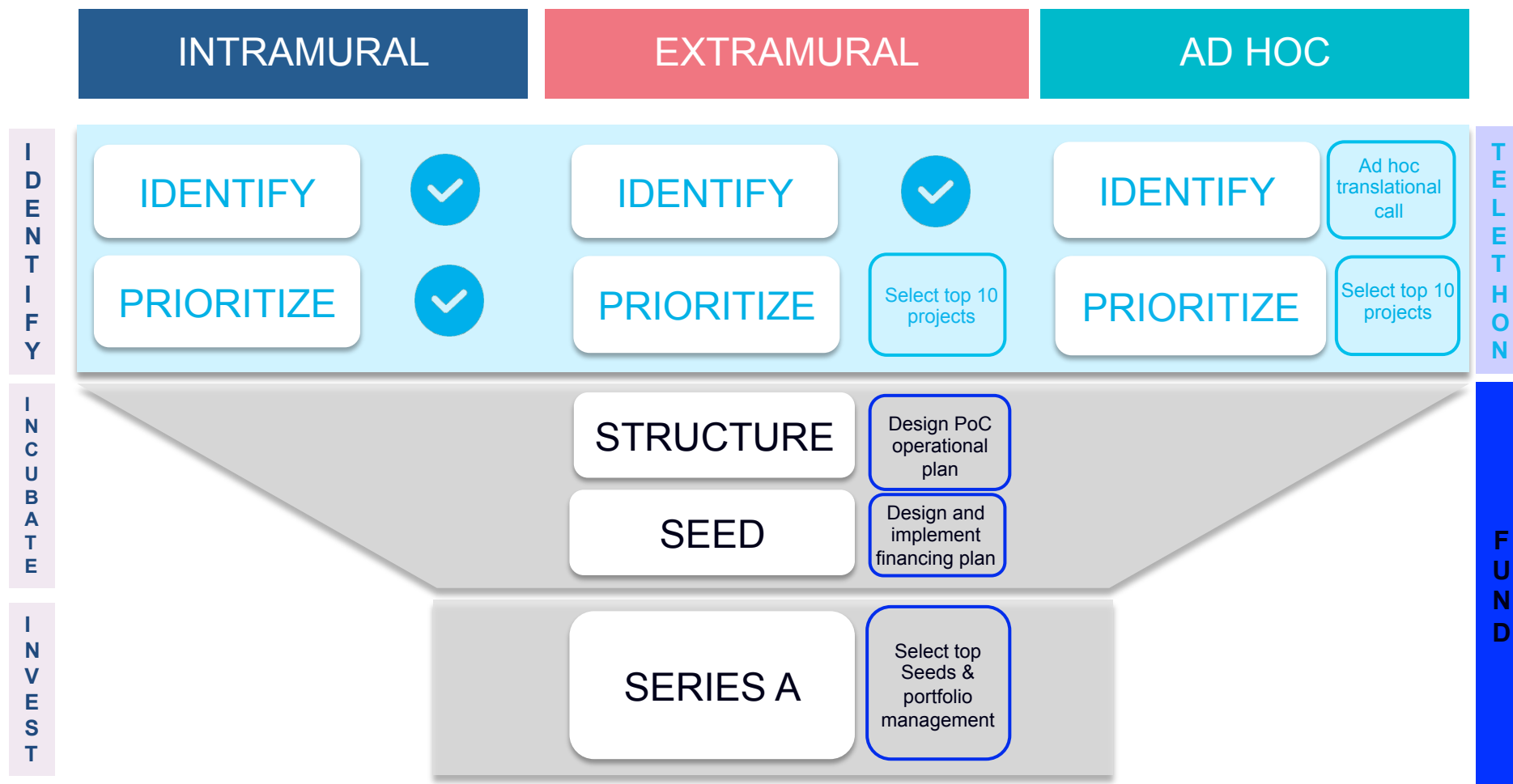
INCUBATE

- ▶ Sharp focus on early stage research projects with the highest commercial potential
- ▶ Bring to Proof of Concept via dedicated seed investments

INVEST

- ▶ Invest in the most promising and successful seed projects
- ▶ Structure broad international Series A
- ▶ Develop management teams with strong domain expertise

The pipeline



Enabling factors for competitive/transformational research



- **Excellent fundamental and pre-clinical research**

- Stringent selection system (funding to max. top 20% proposals)
- Adequate funding
- Monitoring research progression and results



- **Identifying projects with translational potential**

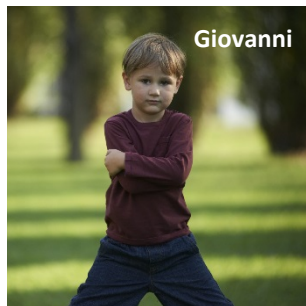


- **Effective translational research**

- Intellectual property protection and technology transfer
- Management of strategic partnership/alliances
- Management of clinical trials
- Management of regulatory affairs
- Competences in drug development

Thank you!

FONDAZIONE



Giovanni



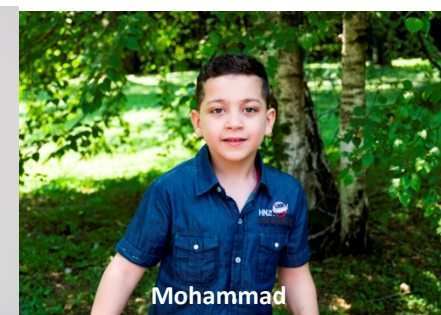
Schana



Ana Carolina



Fabian



Mohammad



Arseny