

Discovery of advanced therapy medicinal products: The Telethon model

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Background

- Telethon is an **Italian charity founded in 1990** by a patient's association, the Italian Union for Muscular Dystrophy (UILDM)
- Telethon **focuses on scientific research** related to rare genetic diseases

MISSION

Advance biomedical research towards the **diagnosis, cure** and **prevention** of muscular dystrophies and other human genetic diseases

VISION

Be the **first foundation to make therapies available** to patients affected by rare genetic diseases

Peer review system

8,000 External reviewers involved since 1990

324 Reviewers in 2013 only (none working in Italy, 140 working in the US)

31 Members of the Scientific Committee (only 1 working in Italy, 19 working in the USA)

Investments

394 Million euro invested in research

2,477 Projects financed, with the participation of 1,547 scientist

445 Diseases studied

Impact

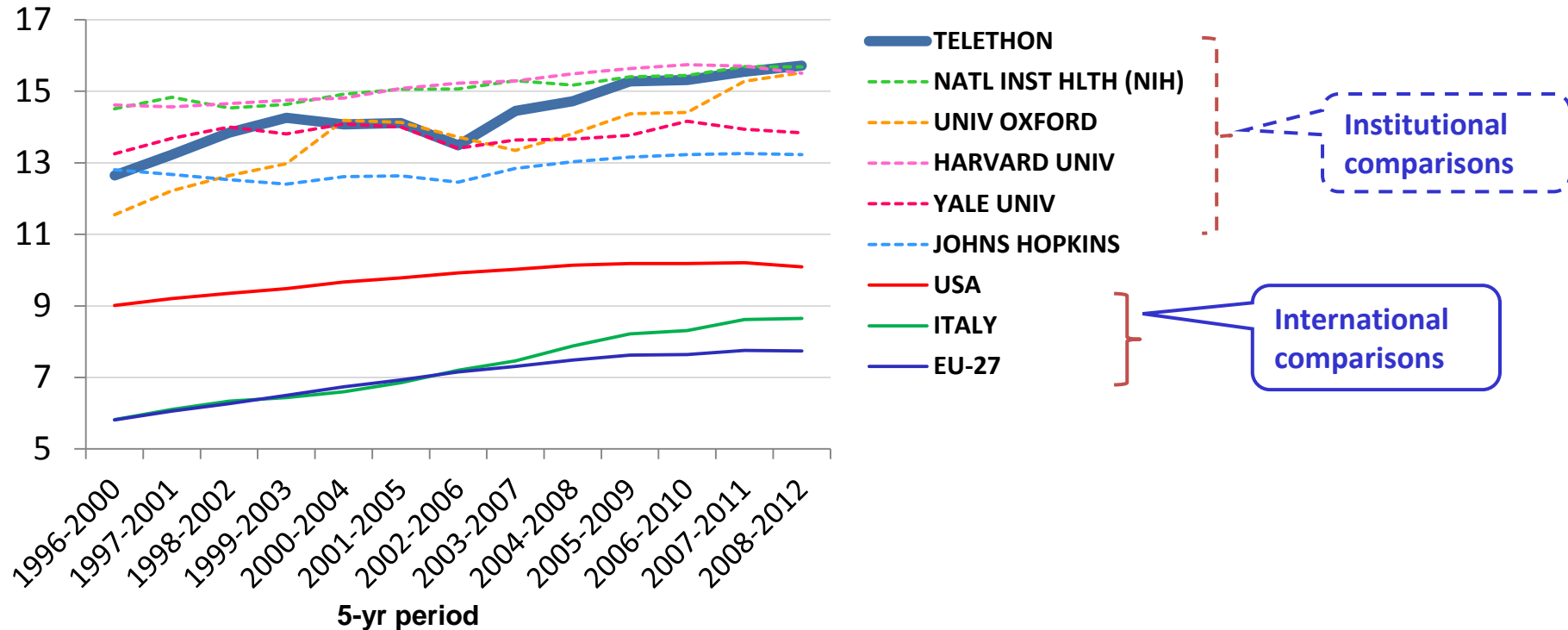
9,386 Scientific articles in international journals

378 Patients involved in clinical trials

3 Diseases treated successfully thanks to Telethon

RESEARCH OUTCOMES: BIBLIOMETRIC ANALYSIS

Cites/paper



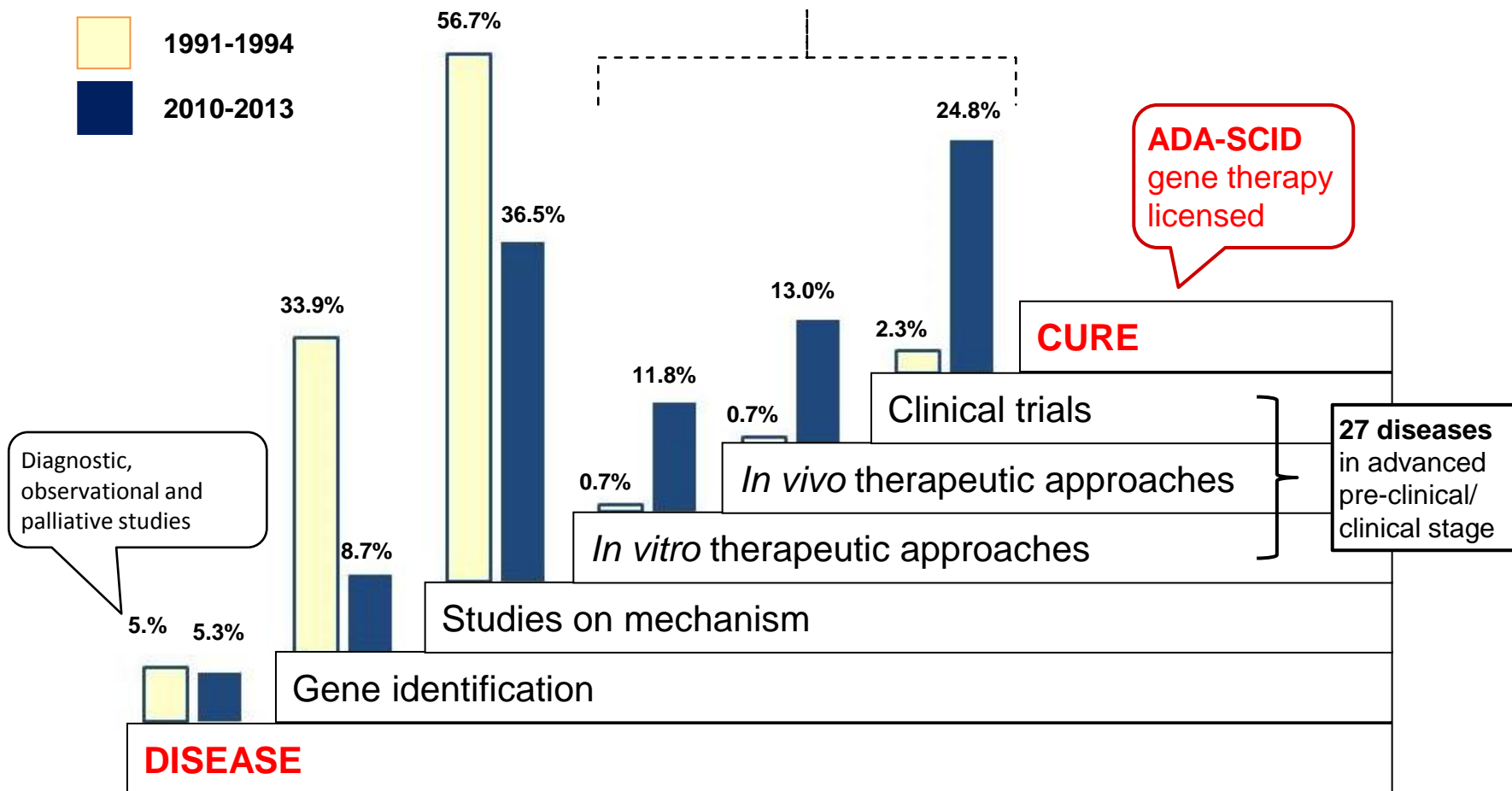
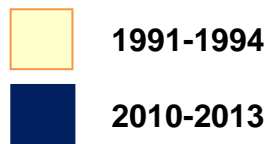
The average number of **citations per paper** in 5-yr time windows shows the **high international impact of Telethon's research results**, in comparison with both Country averages and with representative top level scientific institutes in molecular biology and genetics (<http://www.timeshighereducation.co.uk>).

The analyses regard original articles and reviews in the **6 major biomedical research areas** (biology & biochemistry, clinical medicine, immunology, molecular biology & genetics, neuroscience & behavior and multidisciplinary).

RESEARCH OUTCOMES: PROGRESSION TOWARDS A CURE

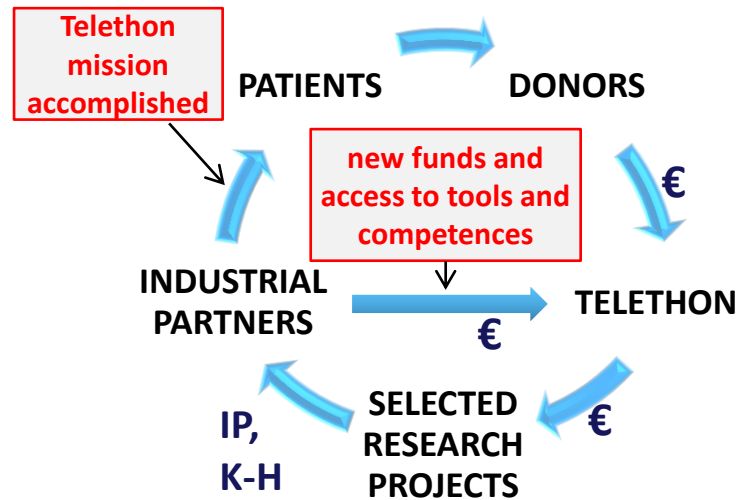
Percent funding distribution

50% research investment in Therapeutic approaches and therapeutic trials in the last three years



Source: TRic database, Telethon, February 2014

Telethon Business Development activities



IP MANAGEMENT

- 23 active patent families
- 81 active patent/patent applications of which 61 included in industrial partnerships

REGULATORY AFFAIRS








- 12 orphan drug designations (ODD) for 9 products:
 - 9 ODD at EMA
 - 3 ODD at FDA

MARKETING AND NEGOTIATION

- 9 sponsored research contracts
- 17 license and option agreements
- 22 biotech and pharma partners

Telethon Business Development activities



	Start year	Institute	Scope
 GlaxoSmithKline	2010	 TELETHON INSTITUTE FOR GENE THERAPY	<ul style="list-style-type: none"> Lentivirus-based ex vivo gene therapy for ADA-SCID, WAS, MLD and 3 other diseases.
 BOMARIN	2011	 TELETHON INSTITUTE OF GENETICS AND MEDICINE	<ul style="list-style-type: none"> Small molecule drug candidates for Lysosomal Storage disorders and neurodegenerative diseases
 Shire	2012	 TELETHON INSTITUTE OF GENETICS AND MEDICINE	<ul style="list-style-type: none"> Gene therapy and small molecule approach for the treatment of Lysosomal Storage disorders and neurodegenerative diseases
Biotech company	2014	 TELETHON INSTITUTE FOR GENE THERAPY	<ul style="list-style-type: none"> Lentivirus-based gene therapy for blood disorders

Conclusion



- Telethon was born to respond to the unsatisfied medical need of rare diseases patients
- The funding of excellent biomedical research is the necessary primary tool
 - a) to unravel pathological mechanisms
 - b) and develop therapeutic approaches
- Strategic portfolio management and partnering with key players involved in the healthcare system are necessary actions to fulfill the promise

Thank you!

