

Evaluating research outcomes and impact: the approach of Telethon Italy



European Forum on Philanthropy and Research Funding

Workshop — by Lucia Monaco, Chief Scientific Officer, Telethon (Imonaco@telethon.it)

Brussels, 5 November 2008

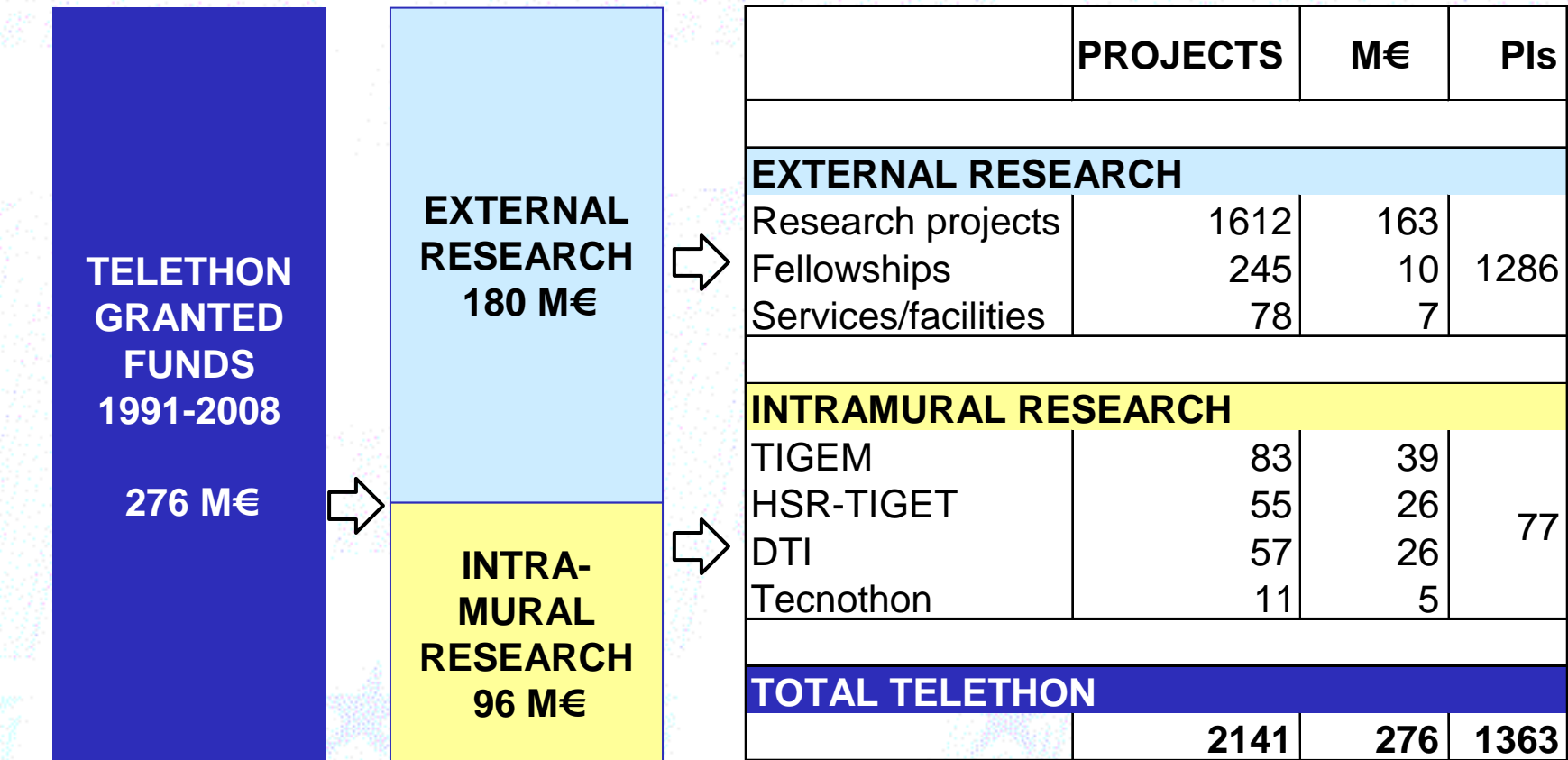
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TELETHON ITALY: A CHARITY FOR RESEARCH ON GENETIC DISEASES

- Telethon Italy is a fund-raising charity founded in 1990 by a patient's association, the Italian Union for Muscular Dystrophy (UILDM).
- **Telethon's mission is to advance biomedical research towards the diagnosis, cure and prevention of muscular dystrophies and all other human genetic diseases**
- As part of its mission, Telethon is also involved in informing patients' associations and in raising awareness on the scientific and social issues related to hereditary genetic diseases

TELETHON'S RESEARCH FUNDING INITIATIVES

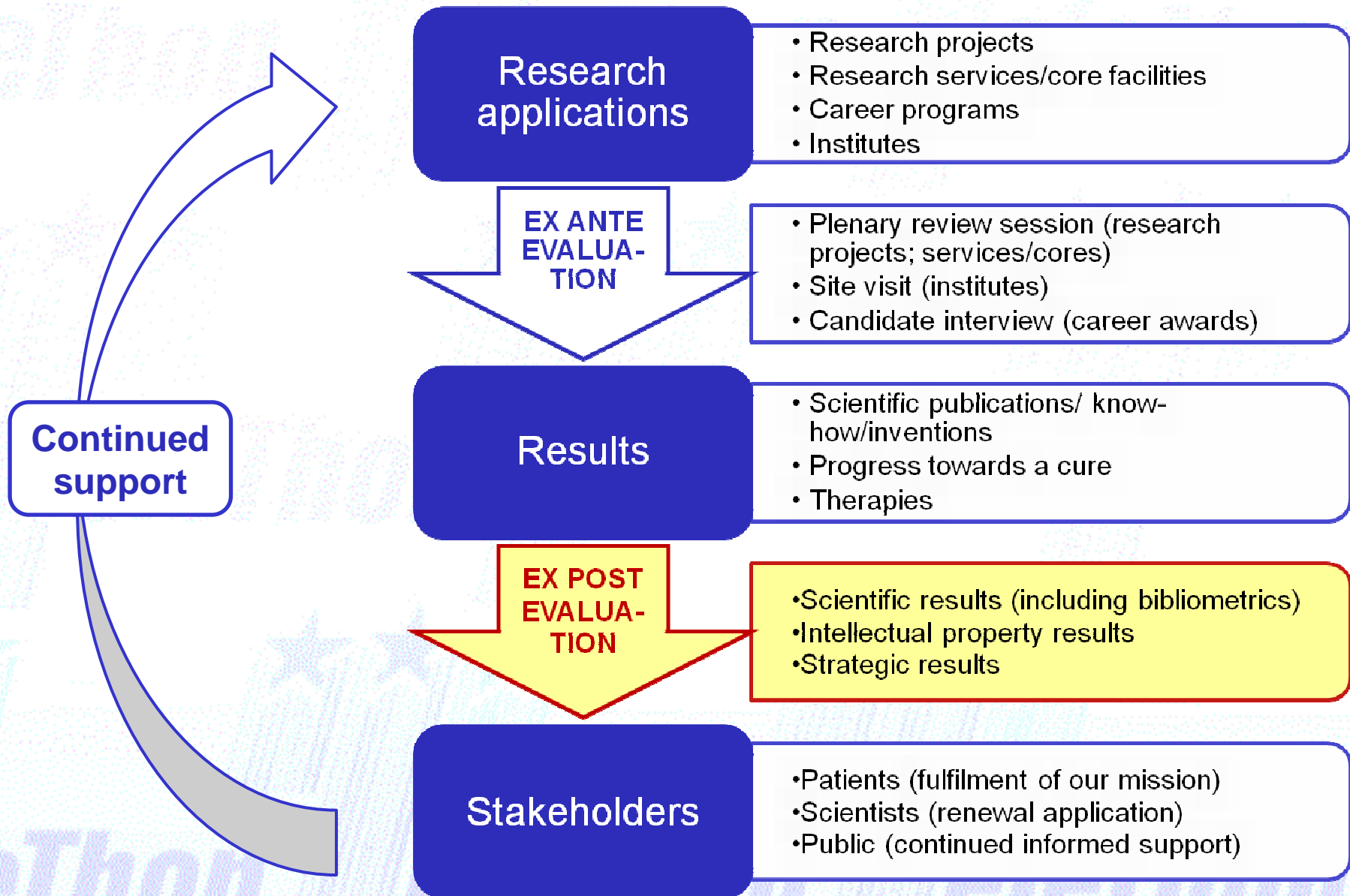
1991-2008



All projects and initiatives are funded through a merit-based selection by peer review

Source: Science Watch Office @Telethon, June 2008
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EX ANTE AND EX POST EVALUATION AT TELETHON



EX POST EVALUATION

- Upon grant completion, all Telethon grantees are required to provide a final report composed of:
 - The **scientific report**
 - The financial report
- The **scientific report** must describe/include:
 - The project's achievements towards the original aims of the project (SCIENTIFIC RESULTS)
 - The progress towards the cure of genetic diseases (STRATEGIC RESULTS)
 - Any divergences from the original plan
 - A lay report for the public (FEEDBACK TO THE PUBLIC)
 - A list of publications and manuscripts (MEASURABLE RESULTS)

ASSESSING FINAL SCIENTIFIC REPORTS

The **final scientific report** is reviewed by

- The **Telethon Scientific Committee**, upon reviewing the grantee's application for further support (if applicable)
 - Scientific evaluation of the final report is one of the evaluation parameters for the renewal application
 - Unfavorable evaluation may hamper the final outcome of the renewal application
- The **Telethon Scientific Office** (in all cases)
 - Progress towards Telethon's goals and publication record are assessed
 - Publications are fed into the Telethon scientific database
 - Assessment of intellectual property results is to be implemented soon

PROBLEMS

- Uneven evaluation by the Scientific Committee
- Difficult assessment of Telethon goal's fulfilment

BIBLIOMETRIC ANALYSIS

- Bibliometric analysis of individual Telethon applicants or grantees is not performed (although the applicant/grantee's publication record is assessed and evaluated as a whole)
- All publications acknowledging Telethon's support are collected in a database
- The database is updated weekly with data from
 - Grantees' information
 - Final reports
 - Interim reports (e.g. Telethon Convention's abstracts)
 - Literature databases' TOC and ad-hoc alerts
- Bibliometric analysis is performed on the whole Telethon publication database
- Telethon's publications are annotated once a year with Thomson's citation data
- Aggregate reports are prepared and compared to national and international data
- Results are used as an ex-post proxy measure of the quality of Telethon's selection of grant applications

PROBLEMS

- Citation data are not freely available
- Comparison to other funding bodies is not possible
- The research's impact on Telethon's goals cannot be inferred from bibliometric data

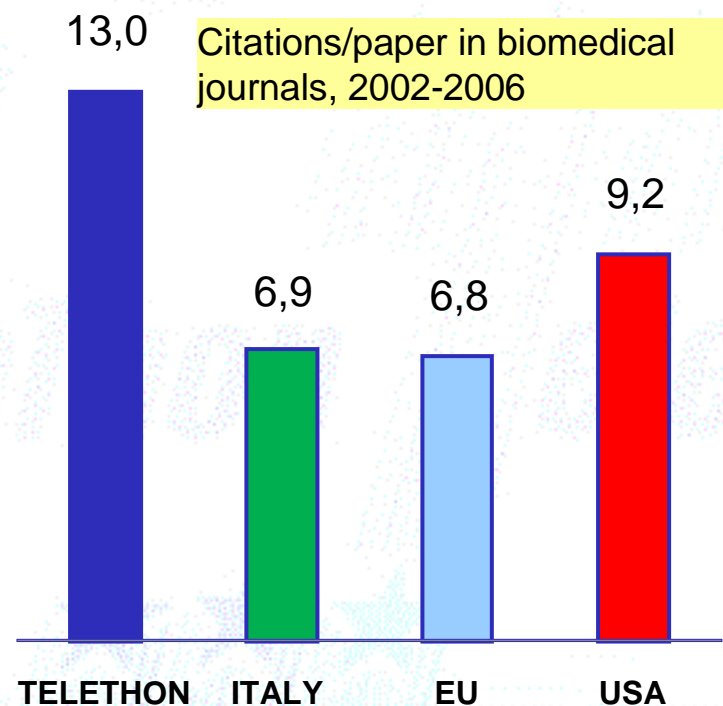
THE OUTCOMES OF TELETHON'S RESEARCH

1. Scientific results
2. Progress towards the cure
3. Therapies

**TELETHON'S
RESULTS**

1. SCIENTIFIC RESULTS – BIBLIOMETRIC DATA

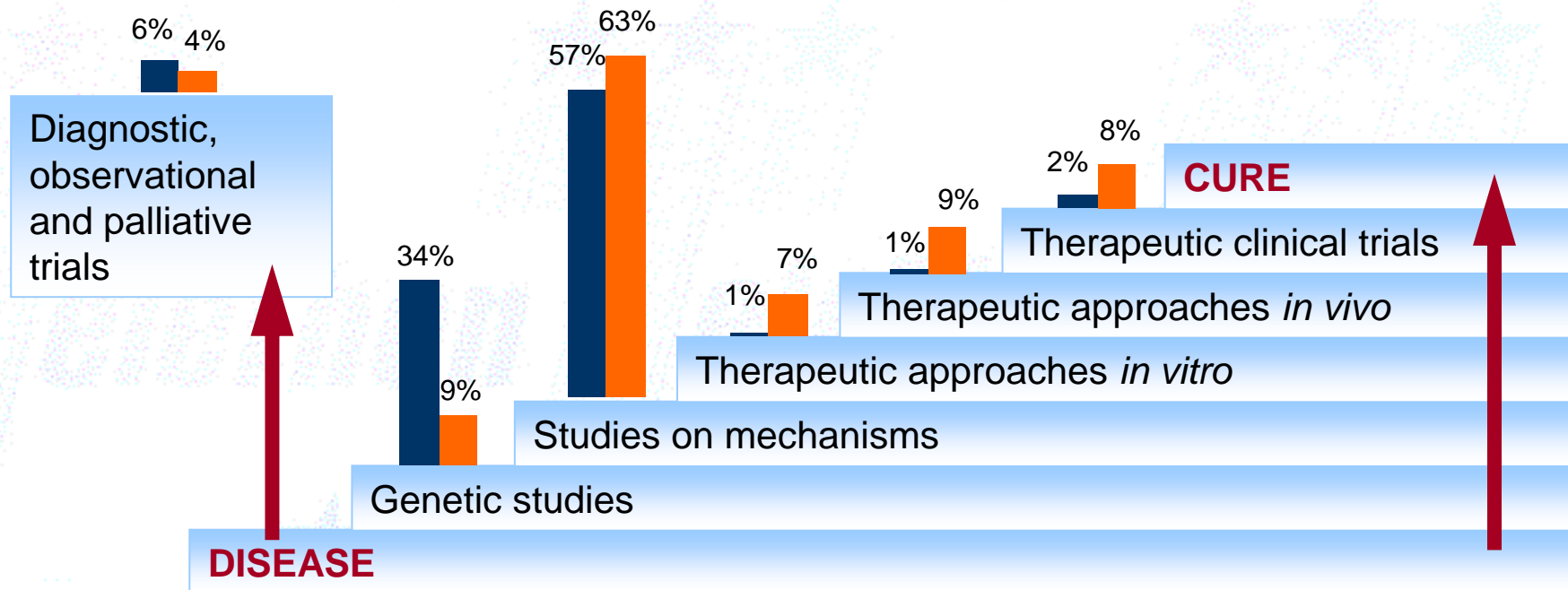
- Over 6200 papers in peer-reviewed journals since 1991
- The impact (average citations per paper) of Telethon papers is higher than for papers published in Italy, the USA and Europe).



Source: Thomson Scientific, Philadelphia

2. PROGRESS TOWARDS THE CURE - THE LADDER OF RESEARCH

A tool for research portfolio analysis and management



Source: Science Watch Office @Telethon, June 2008

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Granted funds

- 1991-1994
- 2004-2007

3. THERAPIES

- Ten children affected by adenosine deaminase severe combined immunodeficiency, **ADA-SCID** have been cured by the first safe and effective gene therapy protocol at the HSR-TIGET institute
- The HSR-TIGET protocol has obtained the Orphan Drug designation from the European Agency EMEA
- Telethon is now applying for registration of the therapy in Europe in order to make it available to all patients



NEXT STEPS

More clinical trials are underway or active:

- Wiskott-Aldrich syndrome (HSR-TIGET)
- Metachromatic leukodystrophy (HSR-TIGET)
- Leber congenital amaurosis (TIGEM)

CONCLUSIONS

- Telethon is a charity funding research to the aim of curing genetic diseases
- To ensure the best results, we apply a rigorous selection of the research initiatives we support
- Ex post evaluation of the research outcomes is mandatory to assess progress towards our goals and to provide our stakeholders with transparent reports
- Our evaluations rely only in part on bibliometric analysis, as successful therapies for genetic diseases constitute the ultimate goal of our activities