

# Telethon funding of biomedical research: from the laboratory to the clinic

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Chiara Cecchi

Technology transfer manager, Telethon Foundation

“Money push vs deal pull”

Moncalieri, October 30-31, 2008

eThon

eleThon

eleThon

“Money push vs deal pull”

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## OUTLINE

- Telethon funding activity
- Aiming at therapies
- The technology transfer at Telethon

### FONDAZIONE TELETHON: SOME INFORMATION 1/2

- A major Italian fund-raising charity - raises and distributes funds for biomedical research on muscular dystrophy and other genetic diseases in universities and non-profit research institutes ([www.telethon.it/english](http://www.telethon.it/english))
- The idea for an Italian Telethon (*Television Marathon*) was inspired by the success of the well-known TV marathon promoted by the actor Jerry Lewis in the USA and the analogous initiative in France. Telethon Italy was founded in 1990 by a patients' association, the Italian Union Against Muscular Dystrophy (UILDM), with the aim of funding research on muscular dystrophies
- Total funds raised since 1990: 348 M€
- Total funds dedicated to scientific research: 276,5 M€
- Total number of publications: more than 6.200; 10% of these in the top 1% scientific journals

*Updated to June 2008*

## FONDAZIONE TELETHON: SOME INFORMATION 2/2

- Telethon research activities include intramural research, grants, career development programs and fellowships
- Telethon awards funds through competitive calls and peer review: all applications undergo peer review by external reviewers selected from the scientific community; final decisions are taken by an International Scientific Committee whose members are leaders in different fields of biomedical research
- The complete transparency of the funds administration is one of the Italian Telethon's main goals
- Telethon is proud to certify that 80 cents out of every Euro raised are directly devoted to research. Only 20 per cent of the money is spent for covering management and the fund-raising costs

## TELETHON'S MISSION

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- We aim to help advance scientific research towards a therapy for muscular dystrophies and other genetic diseases
- We give priority to rare genetic diseases that are neglected by major public and industrial funding
- 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

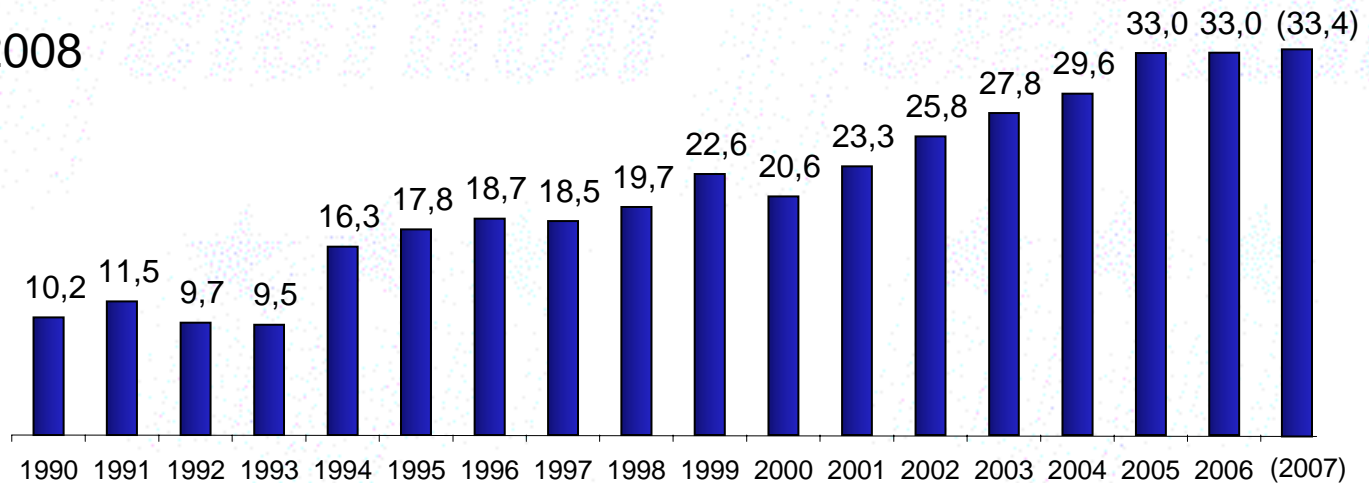
Source: Telethon's Statute, January 2002

# TELETHON'S FUND RAISING AND FUNDING PROGRAMS

## Raised funds

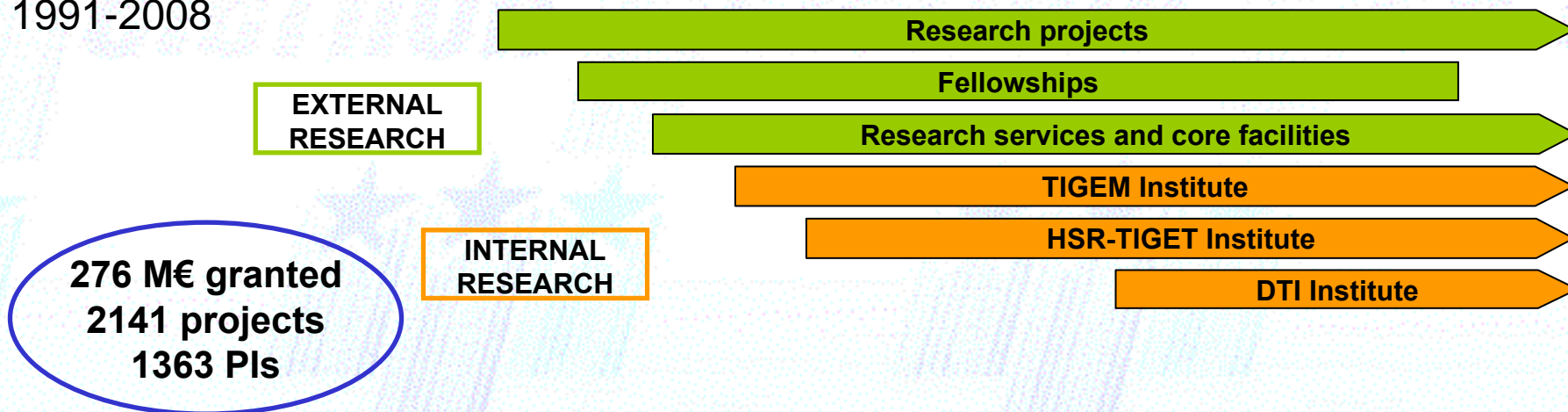
Millions Euro 1990-2008

**Total:  
348 M€**



## Funding Programs

1991-2008



**276 M€ granted  
2141 projects  
1363 PIs**

Source: Telethon Balance Sheet 2007 / Science Watch Office @Telethon, June 2008  
CSS-Moncalieri/30.10.2008/TTTO/CC

## TELETHON NUMBERS 1990-2008

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<b>External Research</b>	<b>No. of Funded Projects</b>	<b>Funds (M€)</b>
Research Projects	1.935	163,1
Fellowships	245	10,0
Services/Facilities	78	7,0
<b>Total</b>	<b>2.258</b>	<b>180,1</b>

<b>Intramural Research</b>	<b>Funds (M€)</b>
TIGEM	38,9
HSR-TIGET	26,3
DTI	25,9
Tecnothon	5,2
<b>Total</b>	<b>96,3</b>

Telethon funds three research institutes and one technical lab

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## INTRAMURAL RESEARCH

1. The **Telethon Institute of Genetics and Medicine** (TIGEM), started in 1994, is located in Naples and is dedicated to disease gene identification, with a particular emphasis on functional studies both *in vitro* and *in vivo*. Director: Andrea Ballabio. About 130 people
2. The **San Raffaele-Telethon Institute of Gene Therapy** (HSR-TIGET), created in 1995, is a joint venture with San Raffaele Institute in Milan and is devoted to the development of gene therapy protocols and clinical trials for inherited diseases (congenital immunodeficiencies, lysosomal storage diseases and insulin-dependent diabetes mellitus). Director: Luigi Naldini. About 65 people
3. The **Dulbecco Telethon Institute** (DTI), founded in 2001, has several locations throughout Italy, and it concentrates on the basic aspects of research on genetic diseases. About 140 people
4. The **Tecnothon**, located in Sarcedo (Vicenza) is a laboratory specialized in the design of prototypes and devices for disabled people. Patented inventions are assigned to companies upon the guarantee of production and distribution of the final product at an “ethical” price

More than 400 genetic diseases have been studied, 80% are monogenic diseases

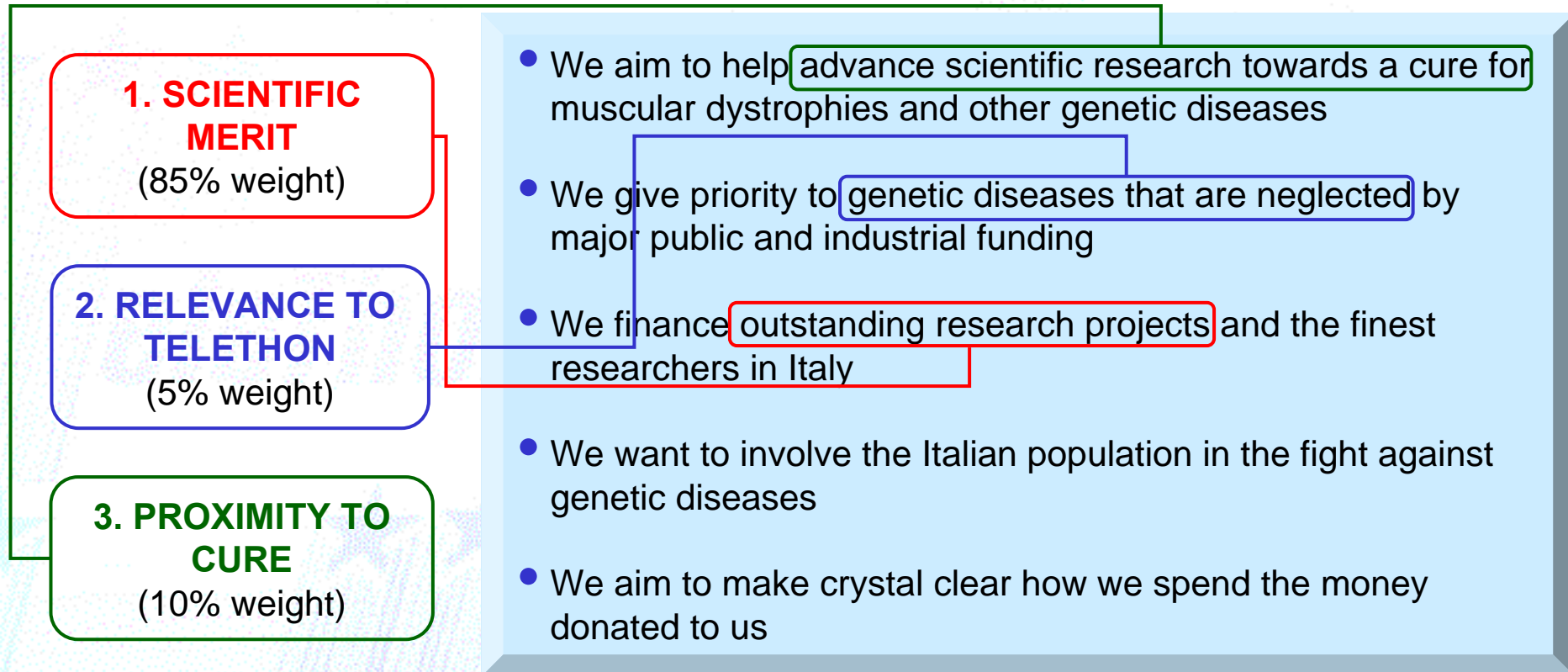
## TELETHON'S MOST FUNDED DISEASES

	DISEASE		GRANTED FUNDS
1	Duchenne muscular dystrophy	€	10.502.842,22
2	Severe Combined Immunodeficiency, ADA	€	7.848.104,49
3	Muscular dystrophies	€	7.197.101,96
4	Amyotrophic Lateral Sclerosis	€	5.621.183,31
5	Diabetes, type 1	€	4.252.284,22
6	Metachromatic Leukodystrophy	€	3.951.953,85
7	Alzheimer disease	€	3.797.234,09
8	Charcot-Marie-Tooth	€	3.182.613,78
9	Wiskott-Aldrich Syndrome	€	3.104.631,22
10	Huntington Disease	€	2.631.673,91
11	Spinal muscular atrophy	€	2.370.881,45
12	Mitochondrial Encephalomyopathies	€	2.302.772,73
13	Cystic Fibrosis	€	2.252.728,52
14	Optic atrophy, type I	€	2.222.524,96
15	Epilepsy	€	2.092.748,36
16	Retinitis pigmentosa	€	2.059.129,49
17	Spastic paraplegia, hereditary	€	1.871.724,15
18	Hypertrophic Cardiomyopathy, familial	€	1.858.478,33
19	Epidermolysis bullosa	€	1.845.966,92
20	Ataxia Telangiectasia	€	1.838.325,01

Careful management of the research portfolio is needed to reach the goals of Telethon's mission

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## TELETHON'S SCORING SYSTEM



In april 2004 Telethon Italy is acknowledged as a top research funding organization

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## IMPACT ON SCIENTIFIC COMMUNITY



ELSEVIER

Eur. J. Cell Biol. 83 (2004); 93–95  
<http://www.elsevier.de/ejcb>



Editorial

**Reviewing in science requires quality criteria and professional reviewers**

Karin Jurkat-Rott, Frank Lehmann-Horn  
Abteilung für Angewandte Physiologie, Universität Ulm, Ulm, Germany

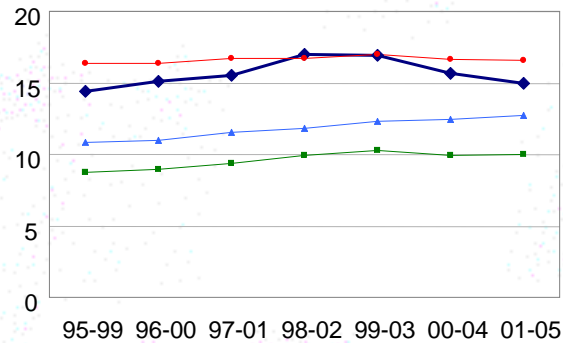


Several research funding organizations have responded to these demands and have developed review criteria and effective review procedures to ensure quality and reduce bias and injustice. Examples are not only big federal research foundations like the NIH but even smaller private ones such as Telethon in Italy specialized on funding research into muscular disorders. However, editors of many scientific journals, even

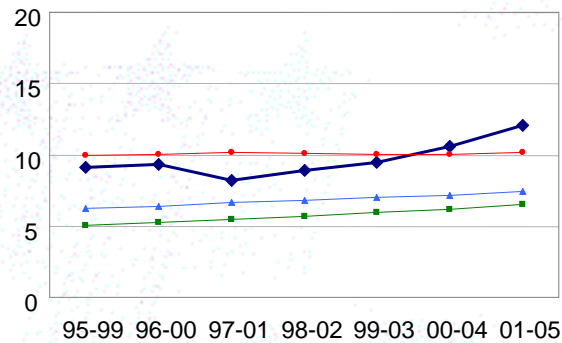
Average citations/paper, 5-year windows, 1995-2005

## IMPACT OF TELETHON PUBLICATIONS

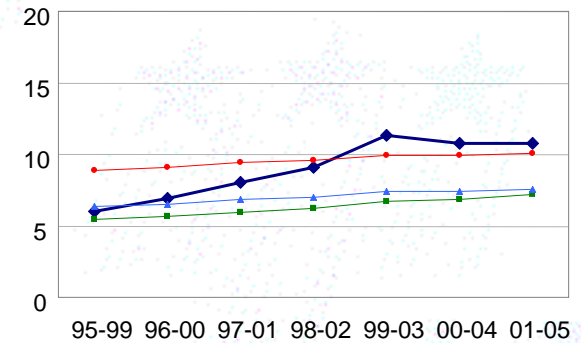
### Molecular Biology & Genetics



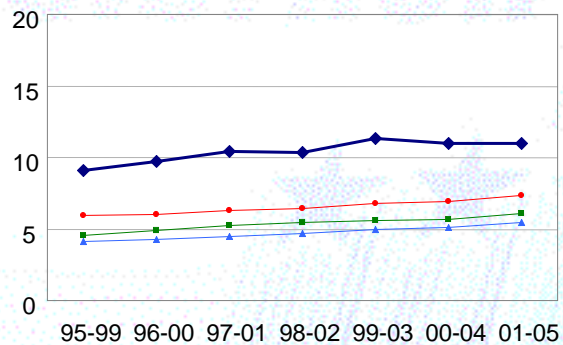
### Biology & Biochemistry



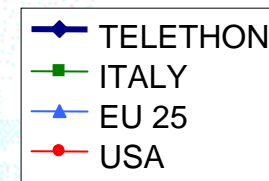
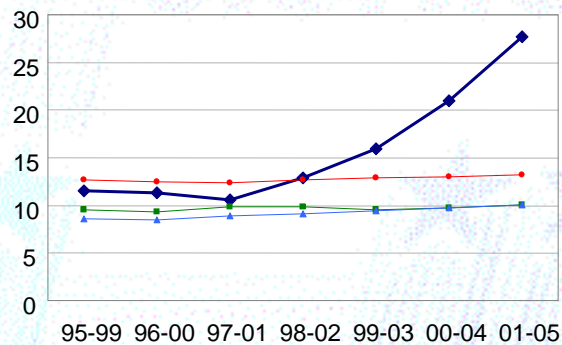
### Neurosciences & Behavior



### Clinical Medicine



### Immunology



Source: Science Watch Office @ Telethon and Thomson-Philadelphia

At the HSR-Telethon Institute of Gene Therapy 11 SCID-ADA children have been cured with gene therapy since 2001

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## ADA-SCID: THE FIRST THERAPY

- Eleven children affected by adenosine deaminase severe combined immunodeficiency, **ADA-SCID**, have been cured by the first safe and effective gene therapy protocol in the world at the HSR-TIGET. Such protocol was recently adopted by the FDA to replace all the ongoing protocols in USA
- Telethon has obtained the Orphan Drug designation from the European Agency EMEA for this protocol
- Telethon is now applying for registration of the therapy in Europe in order to make it available to all patients



### More clinical trials are underway:

- Wiskott-Aldrich syndrome (HSR-TIGET)
- Metachromatic leukodystrophy (HSR-TIGET)
- Leber congenital amaurosis (TIGEM)
- Charcot-Marie-Tooth (external research, Istituto Besta Milan)

The creation of a technology transfer office able to protect intellectual property rights and to transfer these rights to industry is a way to fulfill Telethon's mission

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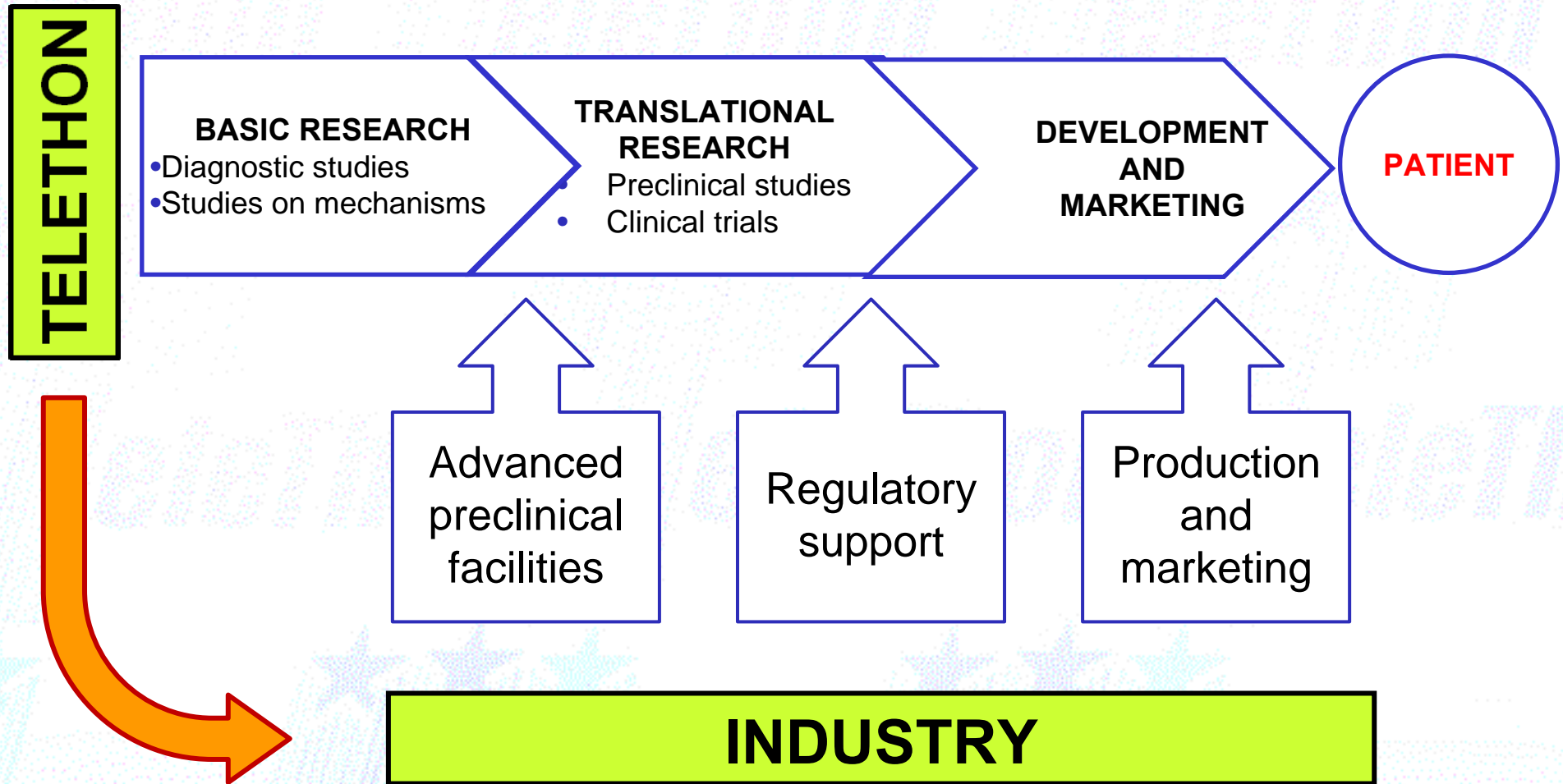
## AIMING AT THERAPIES: THE CHALLENGES OF TRANSLATIONAL RESEARCH

Telethon cannot replicate the experience had with ADA-SCID for other diseases (research funds in 1995-2008: 7,8 M€; development costs 2009-2010: 3,0 M€). For a charity like Telethon, moving research from bench to bedside is too expensive and onerous, and requires increasing costs, dedicated resources and specific expertises.

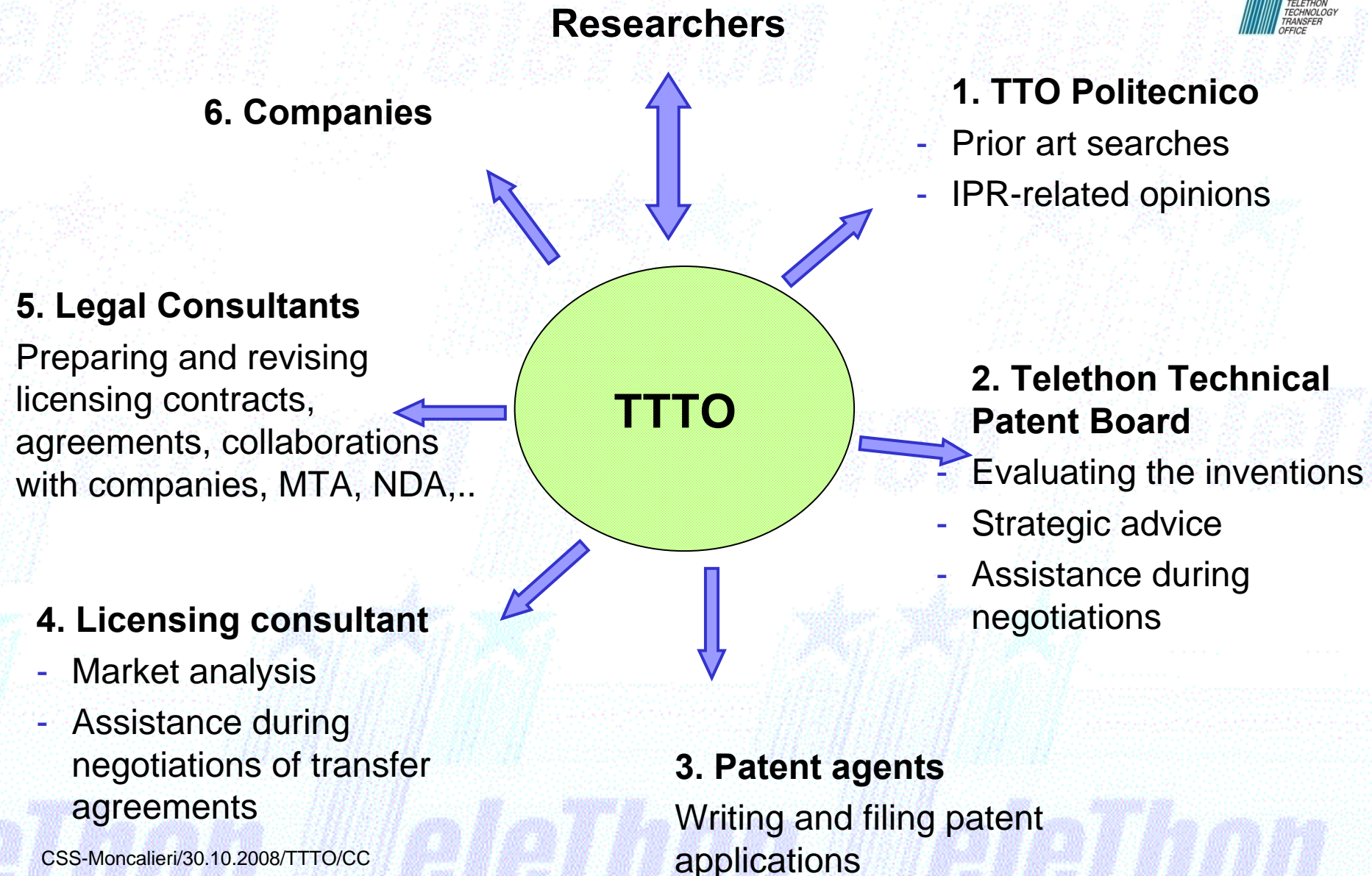


New partnership and new funding models would allow Telethon to face these challenges. In July 2008 Telethon signs a 3 y agreement with **Farmindustria**, the trade association of Italian pharmaceutical industries, with the aim of transferring research results on genetic diseases to clinical development. Collaborations, partnerships, access to facilities, consultancies, developmental programs will be part of this collaborative agreement.

# PARTNERSHIPS FOR THERAPIES



# TTTO: EXTERNAL NETWORK



2005: Fondazione Telethon creates the TTTO



## OVERVIEW OF PATENT PORTFOLIO (TELETHON RESEARCH)

TTTO



1998	2001	2002	2003	2004	2005	2006	2007	2008	TOTAL
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<b>filed inventions</b>	1	1	1	3	1	4	3	1	4	<b>19</b>
<b>live inventions</b>										<b>13</b>
<b>licensed out inventions</b>							2		2	<b>4</b>



<b>TIGEM</b>	1			2	1		1		4	<b>9</b>
<b>HSR-TIGET</b>		1	1			2	2			<b>6</b>
<b>DTI</b>				1		1		1		<b>3</b>
<b>TIDID</b>								1		<b>1</b>



## THE TELETHON TECHNOLOGY TRANSFER OFFICE (TTTO)

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### Key issues - Conclusions

- Funding excellent research results in excellent scientific results and “easier” technology transfer: companies know scientists and their research, projects and results are more innovative and appealing.
- Rare diseases are slowly becoming areas of interest for several companies. The market is small; developmental costs for orphan drugs are comparable to those for drugs for more common diseases.
- Available funds for technology transfer/patenting are not directed to private research-funding foundations like Telethon. Therefore, careful balance between research funding vs investment in TT and related costs (patent filing and maintenance, external consultants, prize to inventors, ...) is mandatory.
- The income from the licensing activity is paying back for the initial investment. More *ad-hoc* funds would allow the TTTO to increase its activity.



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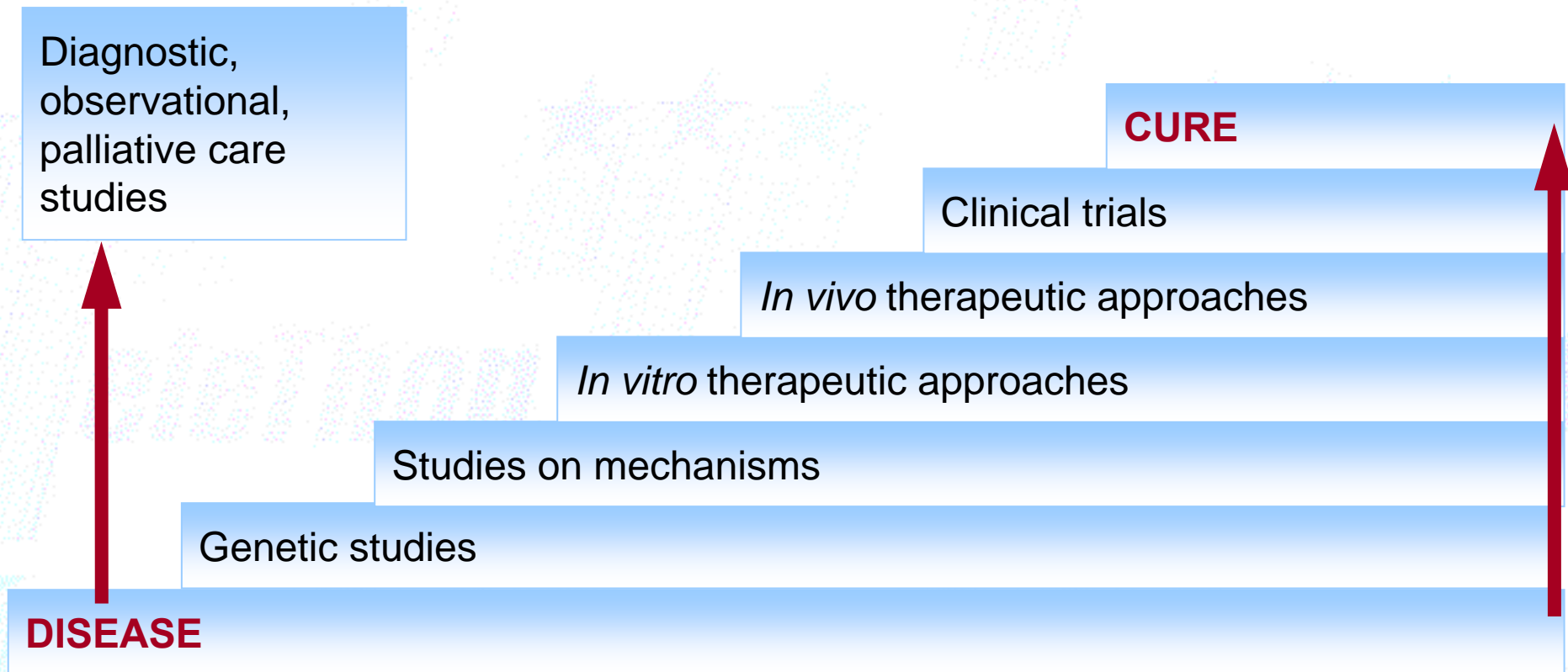
*THANK YOU FOR YOUR ATTENTION!*



To measure whether Telethon funding is moving research towards the cure of genetic diseases, we use a simplified path from disease to cure

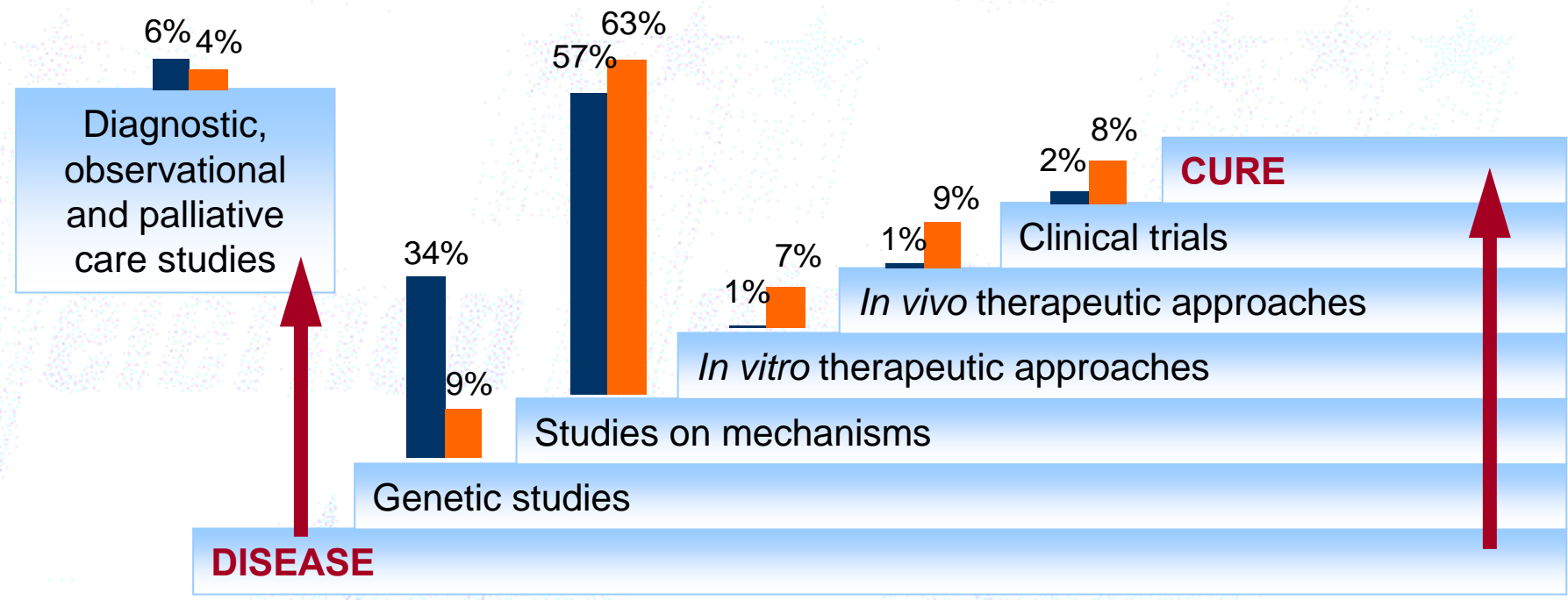
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## THE STEPS OF BIOMEDICAL RESEARCH



A tool for research portfolio analysis and management

## TELETHON'S FUNDS ON THE LADDER OF RESEARCH



Granted funds

1991-1994

2004-2007



## INTELLECTUAL PROPERTY RIGHTS ON TELETHON RESEARCH



*F. Telethon is a private organization funding two types of biomedical research: internal and external research*

**Internal  
Research**

The IPRs related to the invention **belong to Fondazione Telethon.**

**External  
Research**

The IPRs related to the invention **belong to the researcher** i.e. to the employer (University, Research Institute, CNR,..) with whom F. Telethon has to negotiate a percentage of ownership (according to the degree of funding of the project from which the invention has stemmed) (*2008 call for applications*)