How patient organisations can change the game in innovation and drug marketing: the example of AFMTelethon.

Serge Braun – CSO, AFM-TELETHON

AFMTÉLÉTHON
CURE THROUGH INNOVATION

Téléthon

Since 1987, a national, festive and supportive event:

- 30 hr-life TV marathon
- 200,000 volunteers
- 20,000 local events
- 5 million participant
- 1 million donors
  on D-Day
- > 70 partners
  (private companies, organisations)







Budget 2015 :
121,5 M €

AFMTÉLÉTHON
CURE THROUGH INNOVATION

Patient organizations
- serve as disease-specific expert
- reduce bottlenecks/barriers
- a source of innovation

- **Create a scientific ecosystem**
  - **Access to knowledge, expertise , and patients**
  - **Tools, training and experts that would be difficult or time-consuming for pharma/biotech companies to develop** (e.g. in vitro / in vivo assays, outcome measures, biomarkers, clinical study data, DSMB, patient registries, biobanks, disease natural history data, CIC networks)
  - **Resources** (e.g. GMP manufacturing, patient enrolment, training, informed consent forms)

- **Create a regulatory ecosystem**
  - **A voice**: lobbying and reimbursement, ethics and regulatory committees, EU commissions,…

- **Create an economic ecosystem (cost / risk sharing)**

AFMTELETHON
CURE THROUGH INNOVATION



A CATALYST FOR RESEARCH AND THERAPEUTIC INNOVATION

AFMTÉLÉTHON
CURE THROUGH INNOVATION

An integrated environment for therapies of rare diseases

An international network of
- **hundreds of research teams**
- **patients organizations**
- **6000 experts**

- **> 200 R&D projects / y**
- **> 70 fellowships / y** France and abroad
- **> 15 large programs, platforms**
  (project management teams)
- **> 36 clinical trials (25 rare diseases)**

AFMTELETHON INNOVER POUR GUERIR

40%

60%

GENETHON INNOVER POUR GUERIR

220 scientists

Gene therapy of rare diseases (from bench to bed)

INSTITUT DE MYOLOGIE

270 MDs, PhDs, and techs, 50 % AFM, 50 % Hospital / INSERM / UPMC





I-Stem INNOVER POUR GUERIR

85 scientists
50 % AFM
50 % INSERM

Stem cells



Atlantic Gene Therapies Nantes

AFMTELETHON CURE THROUGH INNOVATION

1987:
Telethon launched

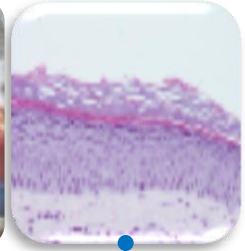


1990-2000:
Scientific revolution



2000-2010:
Medical revolution





2010-2016:
First treatments…





1958:
AFM launched

1st Telethon
1987

1st human genome map
1992

1st POC of gene therapy
2003

1st reconstitution of human skin
2010

36 clinical trials

Gene therapy
Cell therapy
Pharmacology

1990
GENETHON



1996
INSTITUT DE MYOLOGIE



2005
I-Stem
INNOVER POUR GUERIR



2012
GENETHON BIOPROD



A WORLDWIDE NETWORK OF PARTNERS OF EXCELLENCE

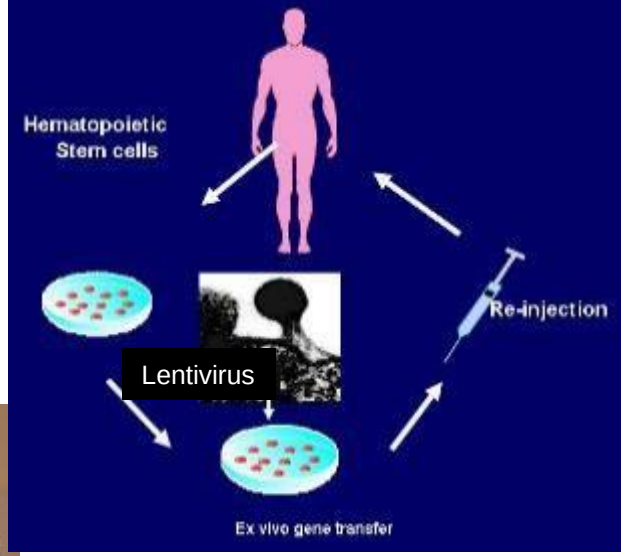


GENETHON
CURE THROUGH INNOVATION

AFMTELETHON
CURE THROUGH INNOVATION

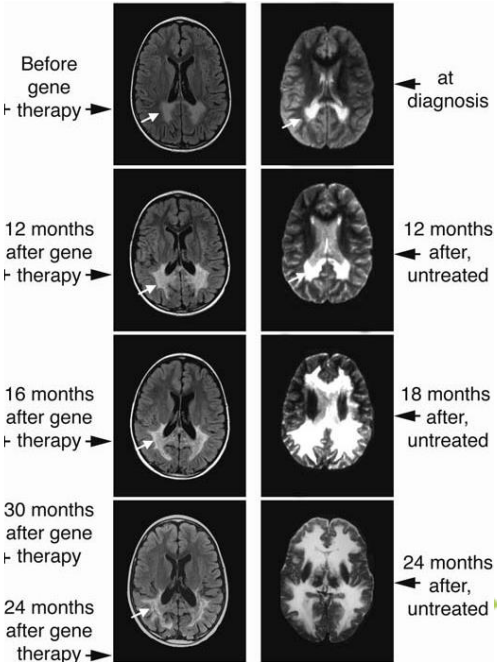






POC
Paving the way for more diseases

- Immunodeficiencies
- Blood disorders
- Adrenoleukodystrophy
- Bone diseases



Gene therapy of X-linked myotubular myopathy



- **Fatal : >50% avant 2 ans**
- **Muscle weakness, respiratory failure**
- **No treatment**



Ana Buj-Bello



Forbes

PHARMA & HEALTHCARE | 3/26/2014 @ 6:00AM | 10,963 views

Gene Therapy's Big Comeback

GOING VIRAL

COMPANIES THAT USE VIRUSES TO REPLACE DISEASE GENES HAVE BEEN BOOMING—AT LEAST WHEN IT COMES TO FUND-RAISING. LOOK HOW MUCH THESE FIRMS HAVE RAISED SINCE THE BEGINNING OF 2013.

COMPANY AIMS TO TREAT	RAISED ($MIL)
GENSIGHT BIOLOGICS GENETIC BLINDNESS	$41
BLUEBIRD BIO "LORENZO'S OIL" DISEASE	$116
AUDENTES THERAPEUTICS X-LINKED MYOTUBULAR MYOPATHY	$30
SPARK THERAPEUTICS GENETIC BLINDNESS	$50
DIMENSION THERAPEUTICS HEMOPHILIA	>$10
EDITAS MEDICINE YET TO BE DETERMINED	$43
JUNO THERAPEUTICS CANCER	$120
CELLADON ADVANCED HEART FAILURE	$51
UNIQURE LIPOPROTEIN LIPASE DEFICIENCY	$92
VOYAGER THERAPEUTICS PARKINSON'S DISEASE	$45
NIGHTSTARX RETINAL DYSTROPHIES	$20

■VENTURE CAPITAL ■INITIAL PUBLIC OFFERING

AFMTÉLÉTHON
CURE THROUGH INNOVATION

Forbes

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Gene Therapy's Big Comeback

GOING VIRAL

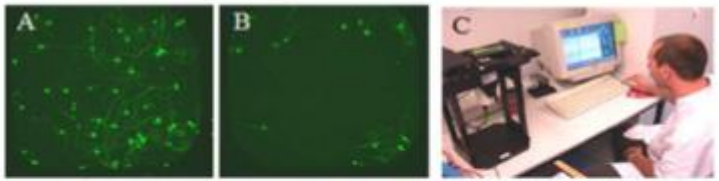
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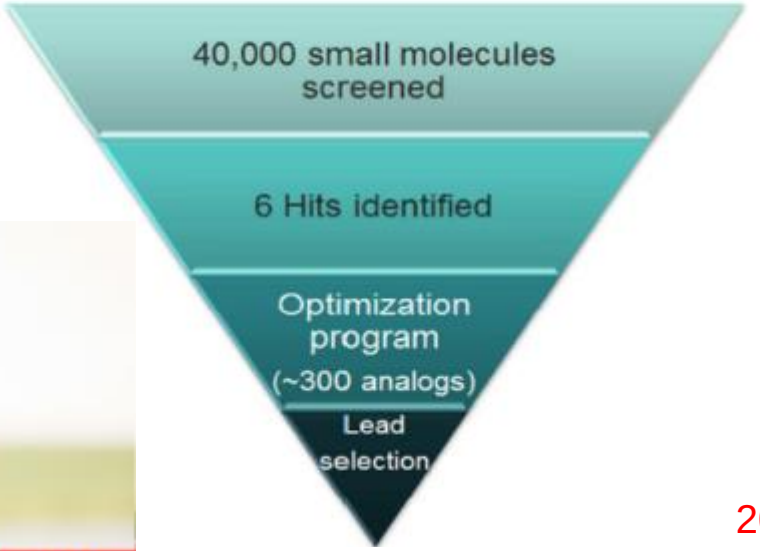
AFMTELETHON
CURE THROUGH INNOVATION

Long-term commitment



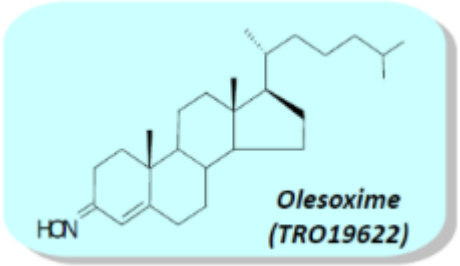
1999

TROPHOS



2004





2014

AFMTELETHON
CURE THROUGH INNOVATION

REUTERS

Roche buys France's Trophos to expand in neuromuscular disease

ZURICH Fri Jan 16, 2015 2:40am

Under the terms of the agreement with Trophos, the French firm's shareholders will receive an upfront payment of 120 million euros ($140 million), plus further payments based on certain predetermined milestones worth up to 350 million euros.



AFM :

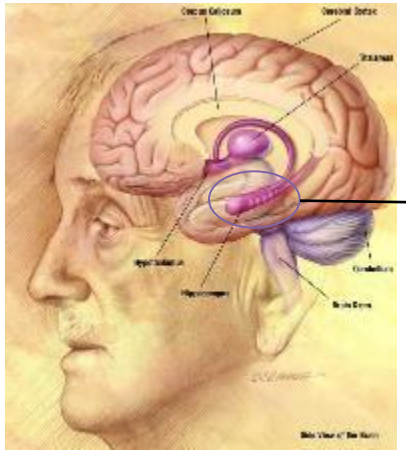
- shareholder (9,6%)
- Exclusive license on Olesoxime

- Garantee patient access + ASAP
  - patient access plan
  - rights back to AFM in case of abandon
- Financial terms

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CURE THROUGH INNOVATION

Win-Win Partnerships

Tech transfer
to discover new drugs
for Alzheimer and schizophrenia



Niche of endogeneous dormant neural stem cells

Stage 1-re-activate proliferation

Stage 2- Promote neuronal production



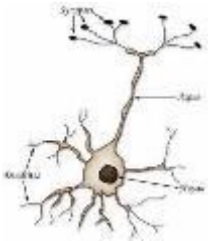
Pionner screening campaigns using human stem cells



Biocell 1800 : plateforme automatisée d'Istem



Biocell 1800 : Bras robotique



AFMTÉLÉTHON
CURE THROUGH INNOVATION

AFMTelethon and the private sector

✓ **Biotechs :**

Trophos, Prosensa, Santhera, Cellectis, Anagenesis, Rhenovia, Pharnext, Transgene, Genset, Gensight,…

✓**Big Pharmas : Roche, + 2 undisclosed top 5**

✓**Spin-off companies :** GenoSafe

AFMTELETHON
CURE THROUGH INNOVATION

AFMTELETHON + BPI France (State Public Bank of Investments) rare disease seed funding

adaire

Chimie Pharma HEBDO



Financements

Un fonds d'amorçage pour les biothérapies innovantes et les maladies rares

50 millions d'euros, c'est la somme initiale injectée dans le « *1er fonds d'amorçage dédié aux biothérapies innovantes et aux maladies rares* ». Créé sous l'impulsion de l'AFM-Téléthon, ce fonds est géré par CDC Entreprises, filiale de la Caisse des dépôts et future entité de bpifrance. L'association de malades apporte 30 M€ tandis que 20 M€ proviennent du Fonds national d'amorçage (FNA), organisme d'État dans le cadre du programme d'Investissements d'avenir. Ce nouveau fonds a pour objectif de « *favoriser les projets thérapeutiques visant à offrir aux patients atteints de maladies rares l'accès à des traitements innovants et permettre l'émergence de traitements pour des pathologies fréquentes à partir des innovations thérapeutiques développées pour ces maladies rares* », selon Laurent Arthaud, directeur délégué de CDC Entreprises et responsable des Sciences de la vie. « *Nous sommes aujourd'hui à la croisée des chemins entre la recherche translationnelle et l'industrialisation à laquelle il faut commencer à penser. Ce fonds est une étape cruciale sur le chemin du médicament* », se félicite Laurence Tiennot-Herment, présidente de l'AFM-Téléthon. Le fonds devrait investir dans 12 à 15 projets pour une durée de 12 ans. Si le budget initial est de 50 M€, les partenaires espèrent pouvoir monter jusqu'à 120 M€, injectés régulièrement, avec « *des tours de tables tous les deux à trois ans* », selon Laurent Arthaud. Le fonds prévoit ainsi d'investir entre 3 et 10 M€ par projet. Pour être éligibles, les entreprises doivent avoir moins de huit ans d'existence, être « *innovantes* » (un critère dépendant de leurs dépenses de R&D ou de l'obtention du label Oseo) et n'avoir pas encore réalisé de levée de fonds. Les investissements devraient concerner majoritairement la France mais pourront être étendus à l'Europe. **A.D.**

50 M€ fund
→ 10 year follow-up

As of today → 5 start-up

AFMTÉLÉTHON
CURE THROUGH INNOVATION

Funding of start-up companies

Company	PAYS	ASSOCIATION	PAY	CAPITAL	FUNDING	YEAR	Colonne1
Aboena	US	15 associations		4 500 000 $		2014	Aquired by Plasmatech Pharma
Acceleron	US	MDA			1 000 000 $	2011	ACE-031
Audentes	US	Joshua Fraze Found. Myotubular Trust				2013	
Capricor Ther.	CA	CureDuchenne	US	206 000 $	1 000 000 €	2015	Cardiospheres
Catabasis Pharm.	US	PPMD	US		100 000 €	2014-2015	edasalonexent
CombinatoRx	US	Charley's Fund + Nash Avery Foundation	US		3 000 000 €	2007	
DART Ther. (Akashi the	US	PPMD	US		300 000 €	2013	
Esperare Foundation	CH	AFM-Telethon	F			2013-	Cardiomyopathies
Halo Ther (Akashi ther	US	Charley's Fund	US		> 4 600 000 $	2012-2014	
IONIS	US	MDA+15 associations	US		1500000 $	2014	DM1. + ALS, Huntington, SMA with GSK, Biogen, Roche, ATL
Lexicon Ther.	US	CureDuchenne	US	5 000 000 $		2013	sphingosine-1-phosphate lyase inh.
Milo Biotech	US	Charley's Fund	US			2015	TG Follistatine
Phrixus Pharm.	US	Coalition Duchenne	US		67 374 $	2012	poloxamer 188 (P-188),
Prosensa	NL	AFM+CureDuchenne, PPMD	EU, US	5 000 000 $	CureDuchenne	2014	
PTC Ther.	US	PPMD+CureDuchenne	US		> 2 550 000 $	2014	
Reveragen	US	Eradicate Duchenne+Ryan's Quest Found.	US		> 1 100 000 $	2013-2015	VBP15
Sanofi/Genzyme	F	MDA	US		120 000 $	2014-2015	Pompe
Sarepta	US	CureDuchenne, Foundation to Eradicate Duchenne+Charley's Fund	US		> 5 500 000 $	2007-2010	
Solid Venture	US	PPMD	US			2014	
Summit	UK	CureDuchenne+5 associations+PPMD	US		1 750 000 $	2011-2012	
Synthena	CH	Ass. Monegasque Myop.	MO		> 10 000 000 €		
Talem Technologies	US	PPMD	US		70 531 $	2015	Exoskeleton
Tivorsan Pharm.	US	PPMD+MDA	US	565 000 +	1 000 000 $	2015	Biglycan

AFMTELETHON
CURE THROUGH INNOVATION

A leading role at each step of the path to the drug

Basic science | **R& D, pre-industrial** | **Industrial**

Public :

ASSISTANCE PUBLIQUE HÔPITAUX DE PARIS

cea INSTITUT PASTEUR

Inserm

Universités

Own labs



Venture philantropy → Start up; Start up; Start up

D

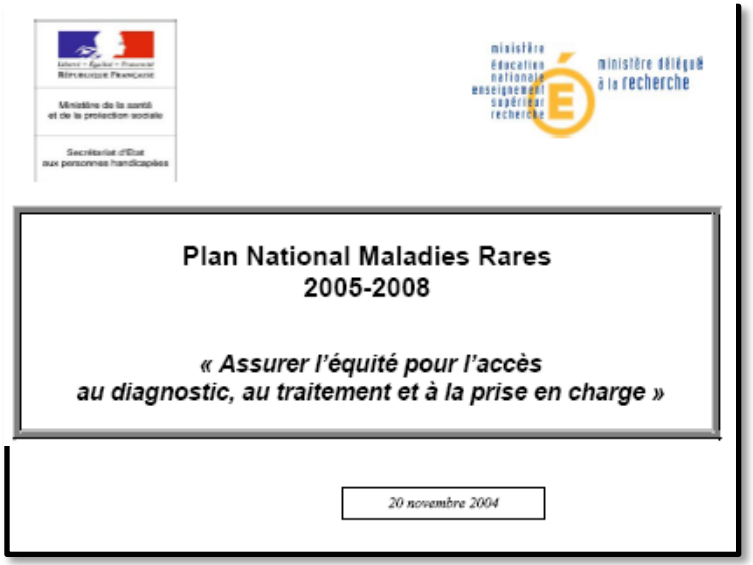
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ÉLETHON
CURE THROUGH INNOVATION

REGULATORY ECOSYSTEM



Ministère de la santé et de la protection sociale

Secrétariat d'Etat aux personnes handicapées

ministère délégué à la recherche

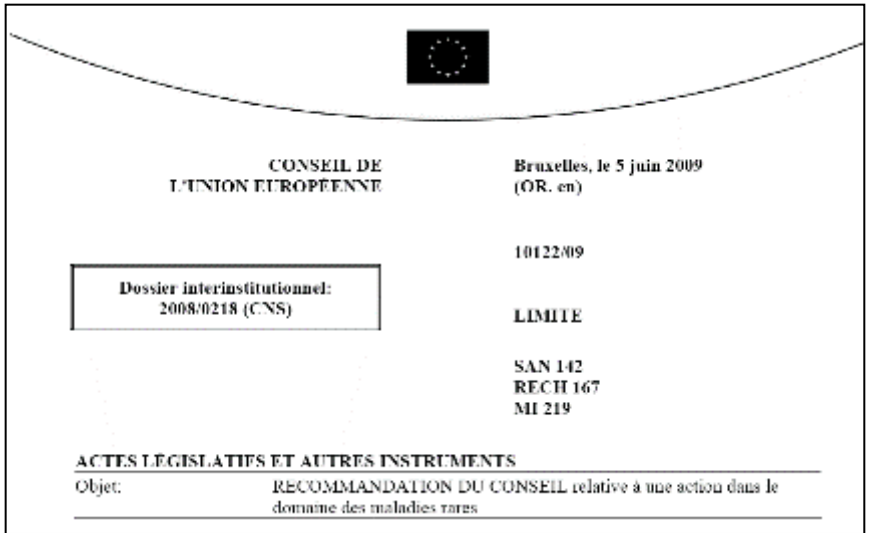
Plan National Maladies Rares
2005-2008

« Assurer l'équité pour l'accès au diagnostic, au traitement et à la prise en charge »

20 novembre 2004

Rare Diseases National Plans

+ Orphan Drug status (via Eurordis)



CONSEIL DE L'UNION EUROPEENNE

Bruxelles, le 5 juin 2009
(OR. en)

10122/09

Dossier interinstitutionnel:
2008/0218 (CNS)

LIMITE

SAN 142
RECH 167
MI 219

ACTES LEGISLATIFS ET AUTRES INSTRUMENTS

Objet: RECOMMANDATION DU CONSEIL relative à une action dans le domaine des maladies rares



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1995
1998>2010
2011

genopole®
RÉUSSIR ENSEMBLE EN BIOTECHNOLOGIES

TÉLÉTHON
FIGURE THROUGH INNOVATION