WHAT CAN INFLUENCE PATIENTS ENGAGED IN HTA?

Issue Panel 11544: Patient organisation conflicts of interest. Do HTA bodies really need to know?

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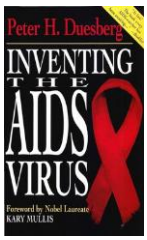

What can influence our opinion?

- **KOL**
  - Key opinion leaders e.g. your scientific committee: Intellectual conflict of interest
- **Politics, religion**
  - Influence of religions on our opinion vis à vis embryologic stem cells? Pre-natal screening?
- **Involvement in research**
  - Patient organisation involved in research – initiating first phase, funding some aspects Participatory conflict of interest
- **Social influencers**
  - Anti-vaccines?
  - Animal research opponents?
- **Funding, career**
  - Pharma?
  - Government?
  - Health insurers?
  - Applying to a job?

Financial or career conflict of interest

We, the undersigned, declare no interests neither in the capital of the marketing authorisation applicants (MAA) "Pharmion Ltd., Cambridge, UK" and "Laphal Developpment, France" or in the capital of any local or international branch of the company. As individuals, we did not work for the applicant, nor did we receive training. Any other financial interests (e.g. unconditional grants) received by our organisations from the pharmaceutical industry and contacts with MAA are listed in annex I of this document.

- The context: MAA for thalidomide

Organisation of victims of thalidomide and patients with multiple myeloma were negotiating a Pregnancy Prevention Programme (PPP), condition for a marketing authorisation

- EURORDIS was leading the negotiations

A PPP was drafted, a common position was reached by all 19 groups

But 6 did not sign the agreement

- EURORDIS tried to understand why

And asked groups to declare their interests vis-à-vis the company

The EMA adopted a policy for organisations
(there was one for experts)

3

Patients' organisations as co-developers: we're in 2021
The existence of PO = determinant for success of R&D

- **Sanfilippo disease (A and B)**
  - **15 patient groups** gave a capital of $4,5 million to create Abeona therapeutics to develop a gene therapy
  - **Acquired** by another company for $45 million
- **Cystic fibrosis**
  - **US CF Foundation (CFF) granted** $150 million to Aurora Biosciences, later acquired by Vertex with royalty rights to develop series of products
  - **Investment fund Royalty Pharma** purchased royalty rights for $3,3 billion to CFF
- **Spinal muscular Atrophy**
  - **AFM-Telethon/Genethon** developed gene therapy vector
  - Zolgensma now commercialised by Avexis with an agreement
- **HIV prevention**
  - **AIDES** received € 500,000 from Gilead for a support programme for the Ipergay trial, as PreP for HIV infection

Société	Association		Capital	Subventions	Année
Anagenesis Biotechnologies	FR AFM	FR	206 000 €	200 000 €	2013
Capricor Therapeutics	CA CureDuchenne	US	1 000 000 $		2015
Catabasis Pharmaceuticals	US Parent Project Muscular Dystrophy	US		na $	2014
Catabasis Pharmaceuticals	US Parent Project Muscular Dystrophy	US		100 000 $	2015
Cellgene	CA AFM	FR	336 000 €	1 379 000 €	2006
CombinatoRx	US Charley's Fund + Nash Avery Foundation	US		3 000 000 $	2007
DART Therapeutics	US Parent Project Muscular Dystrophy	US		300 000 $	2013
EspeRare Foundation	CH AFM	FR		na €	2013
Halo Therapeutics (Akashi Therapeutics)	US Charley's Fund	US		1 548 000 $	na
Halo Therapeutics (Akashi Therapeutics)	US Charley's Fund	US		2 011 000 $	na
Halo Therapeutics (Akashi Therapeutics)	US CureDuchenne	US		na $	2012
Halo Therapeutics (Akashi Therapeutics)	US Parent Project Muscular Dystrophy	US		500 000 $	2014
Halo Therapeutics (Akashi Therapeutics)	US Parent Project Muscular Dystrophy	US		100 000 $	2012
Halo Therapeutics (Akashi Therapeutics)	US MDA + 15 associations	US	1 500 000 $		2014
Lexicon Therapeutics	US CureDuchenne	US	5 000 000 $		2013
Prosius Pharmaceuticals	US Coalition Duchenne	US		67 374 $	2012
Prosensa	NL AFM	FR		3 500 000 €	2003
Prosensa	NL CureDuchenne	US	5 000 000 €		2014
Prosensa	NL Parent Project Muscular Dystrophy	US		200 000 $	2014
Prothelia	US Parent Project Muscular Dystrophy	US		46 000 $	2009
PTC Therapeutics	US CureDuchenne	US		na $	na
PTC Therapeutics	US Parent Project Muscular Dystrophy	US		50 000 $	2010
PTC Therapeutics	US Parent Project Muscular Dystrophy	US		2 500 000 $	2004
ReveraGen Biopharma	US CureDuchenne	US		na $	na
ReveraGen Biopharma	US Parent Project Muscular Dystrophy	US		750 000 $	2015
ReveraGen Biopharma	US Parent Project Muscular Dystrophy	US		49 991 $	2013
ReveraGen Biopharma	US Foundation to Eradicate Duchenne	US		250 000 $	2015
ReveraGen Biopharma	US Ryan's Quest Foundation	US		50 000 $	2015
Sanofi	US MDA	US		60 000 $	2014
Sanofi	US MDA	US		60 000 $	2015
Sarepta Therapeutics	US CureDuchenne	US		250 000 $	2010
Sarepta Therapeutics	US Foundation to Eradicate Duchenne	US		250 000 $	2010
Sarepta Therapeutics	US Charley's Fund	US		2 450 000 $	2007
Sarepta Therapeutics	US Charley's Fund	US		3 000 000 $	2009
Solid Ventures	US Parent Project Muscular Dystrophy	US		na $	2014
Summit	UK CureDuchenne + 5 associations	US		1 500 000 $	2012
Summit	UK Parent Project Muscular Dystrophy	US		250 000 $	2011
Synthena	CH Association Monégasque Contre les Myopathies	MO		na €	na
Talem Technologies	US Parent Project Muscular Dystrophy	US		70 531 $	2015
Tivorsan Pharmaceuticals	US Parent Project Muscular Dystrophy	US		565 000 $	2012
Tivorsan Pharmaceuticals	US MDA	US		1 000 000 $	2015

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PATIENTS WITH POSSIBLE CoI INVOLVED IN HTA

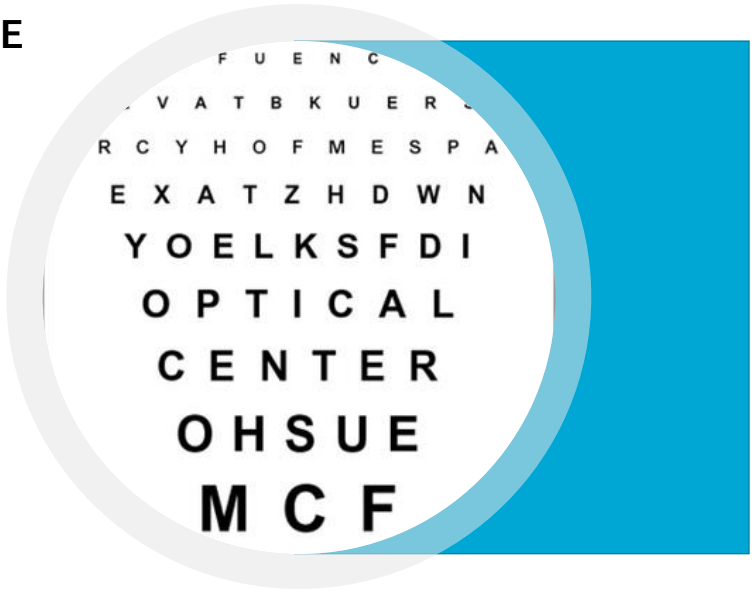
Leber Hereditary Optic Neuropathy

Blindness at a young age, within weeks

Patients consulted by HTA body (SMC)

Including from organisations funded by the developer – which they declared

- Refuted claims of "vision recovery", but "vision improvement"
- For them, price was too high relative to clinical benefits
- Helped authorities to negotiate price: patients and doctors were on same line



To minimise risks

•1

- Adopt strong policies

(e.g. *Code of Practices guiding the Relations between Patients Organisations and the Healthcare Industry*)

•2

- Different roles

Those in contact with industry and representatives consulted by authorities could be distinct persons within the organisations

•3

- Be transparent

Information on funding / revenues available and open for public scrutiny

Still too rare

•4

- Diversify sources, avoid dominant positions
- *A a reserve fund in case you have to end relations with a funder*
- *% of revenues from 1 company should not be too high*

•5

- Clearance

When planning to be consulted by HTA, stop relation with company long ahead of the consultation

CODE OF PRACTICE BETWEEN PATIENTS' ORGANISATIONS AND THE HEALTHCARE INDUSTRY (here)

•1	•2	•3	•4	•5
• Be transparent	• Communicate	• Diversify	• Do not promote	• And also
Patients groups should at all times remain open, honest and transparent concerning the amounts and sources	Annual reports and websites, should clearly illustrate such information and be fully accessible. Indicate % of revenues each source represents	To avoid undue reliance on any particular company, funds should be balanced and diversified as much as possible to guarantee independence	Patient organisations must ensure that none of their activities can possibly be associated with promotional activities	Patient groups and their representatives must refuse to be quoted in industry press releases about a product (authorised or R&D)

To conclude

Declarations come with assessment rules: both should be straightforward, not too complex, not arbitrary. Different rules for patients as experts and for organisations

When assessing CoI risks: proportionate scale of measures (exclusion, restrictions…) – risk not the same for HTA scientific advice / early dialogue or for assessment/appraisal

Policies should be ideally co-developed, or concerted (reality check) – Know each other

Being part of the decision making imposes responsibilities on us – and legal liability

EURORDIS RARE DISEASES EUROPE

Thank you for your attention

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EURORDIS.ORG

https://www.eurordis.org/financial-information-and-funding#tabs-4

Acknowledgements | **Revenue** | Expenses | Corporate revenue

EURORDIS Revenue 2017

Expand All Collapse All

Revenue	Amount (€)	Percentage
⊞Patient Organisations	882 796	16%
⊞Individuals	1 038 157	19%
⊞European Commission	1 392 730	25%
⊞Corporates	1 788 093	32%
⊞Not for Profit Organisations	25 000	0%
Event Fees	112 721	2%
⊞Miscellaneous	354 367	6%
Sub-total	5 593 863	100%
Recovery of provisions	24 350	
Total Revenue	5 618 213	

Acknowledgements | Revenue | Expenses | **Corporate revenue**

EURORDIS Corporate Revenue 2017

Expand All Collapse All

Pharmaceutical & Biotechnology Companies

Company	Amount (€)	% of Revenue
⊞**ABLYNX**	**10 000**	**0,18%**
⊞**ACHILLION**	**26 000**	**0,46%**
⊟**ACTELION**	**45 000**	**0,80%**
EURORDIS Black Pearl Awards	35 000	0,63%
EURORDIS Round Table of Companies	10 000	0,18%
⊞**AEGERION**	5 000	0,09%
⊞**AGIOS**	5 000	0,09%
⊞**AKCEA**	**10 000**	0,18%
⊞**ALEXION**	**25 000**	**0,45%**
⊞**ALNYLAM**	**35 000**	**0,63%**
⊞**AMGEN**	**5 000**	**0,09%**
⊞**AMICUS THERAPEUTICS**	**15 000**	**0,27%**
⊞**ASTRAZENECA**	**10 000**	**0,18%**

BE OPEN, transparent

Better to declare more than not enough

EXPLAIN

Where you're coming from

STATE YOUR OWN

Conflicts. Your credibility will benefit.
Declaring your interests openly makes your credibility

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