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Beyond Orphan Drugs: Cooperation of a Biopharmaceutical Company with other Rare Diseases' Stakeholders

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Conflicts of Interest / Disclaimer

I am currently employed by Genzyme, now a Sanofi Company, and have been since 1993

The views and opinions expressed in this presentation are solely my own and do not necessarily represent the official policy or position of Genzyme, Sanofi or any of its affiliated companies

Focus vs. Mission

The **primary focus** of any biopharmaceutical company is to provide **safe and effective drugs** to **patients**

However, its **mission** can -and desirably should- extend **beyond** this goal

The special case of Rare Diseases

Besides the scarcity of specific therapies, rare disease patients and their families must endure:

- * **long and arduous diagnostic processes,**
- * **haphazard disease management,**
- * **administrative hurdles** to access disability and other social benefits (readily available to those with more prevalent diseases)

Most of these issues can, however be traced to **insufficient:**

- * **medical knowledge**
- * **social awareness**

not only about their specific disease, but also about the complexities of suffering a rare disease in general

Thus, these are obvious areas where a **multi-stakeholder, cooperative approach** can be most effective

A Rare Diseases Pioneer

Genzyme not only developed some of the first & most innovative orphan drugs, it also pioneered many global initiatives in the area of rare diseases, with various **patient and family-oriented resources**, as well as **partnerships and support to patients associations**

Some of its best known global activities include:

- * the “**Expression of Hope**” program
- * the **Patient Advocacy Leadership (PAL) Awards**
- * and extensive **Free Drug** programs



Announcing the Launch of Expression of Hope III
A Global Program of Inspiration and Awareness Featuring Works of Art
by the Lysosomal Storage Disorder Community

Gaucher Program Saves Lives in Egypt, Advances Disease Treatment

January 29, 2013

In 1999, Project HCFE began to implement the Gaucher Disease Initiative in Egypt in partnership with Genzyme Corporation, to relieve the suffering and prevent the untimely death of people affected by the rare genetic disease. Since the program's beginning, Genzyme has donated more than 30 million units of Genzyme (miglustate for injection) the drug used to treat the incapacitating and often deadly disease, and helped to improve the lives of more than 300 children. As Genzyme and Project HCFE commit to another year of the successful partnership, it is a good time to reflect upon the success of the program.

"In 1998, when we began developing the program, no one with Gaucher Disease had ever been treated in Egypt," says Dr. Reda Mansour, Chief of Paedry for Project HCFE in Egypt. "Now there are seven centers for treatment across the country and seasoned clinical specialists who are experts in the treatment of the debilitating disease. This program has not only saved children's lives, it has built capacity and strengthened the local health care system in Egypt and contributed to the treatment of Gaucher Disease around the world."



A more local (mainly Spanish) initiative

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www.fundaciongenzyme.es

General Statutory Goal:

To improve the knowledge, diagnosis and management of diseases of all kind, especially those with a low prevalence and those with unmet medical needs, fostering research, technical and scientific progress as well as promoting dissemination and open exchange of information in these fields, in order to improve healthcare professionals' skills and patients' management and quality of life.

Our three main axes of activities

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Research support

Continued Medical Education

**Patient Empowerment
& Social Awareness**

Research support

Six annual “open calls” for grants

- * Scientific (basic or applied), Medical or Social Research
- * awarded by a Committee led by reputable specialists in the field
- * 10,000€ each (2/3 upfront, 1/3 upon project completion and submission of final report)
- * 1-2 years research projects, not cumulative

Outside these periodic calls, direct institutional R&D support requests are assessed by the Foundation’s Board (supported, when necessary, by additional external experts) and funded according to availability

Projects of possible commercial interest are re-directed to the appropriate Corporate Departments (Medical, Business Development)



Continued Medical Education

Covered through various channels:

- * on-line courses
 - * independently designed by leading specialists
 - * certified by the competent academic board (CFCPSCM): 7.7 credits
- * technical & financial support to specific courses, symposia, congresses, or other educational activities carried out by medical/scientific societies and/or academic institutions
- * electronic media (free, but with required suscription, i.e., no spam)
 - * quarterly newsletter
 - * info about related events & activities (our own or 3rd. parties)

For those less experienced, basic disease information is also hosted on our website, including relevant publications and pre-designed links to key public institutions (NIH's Clinicaltrials.gov and PubMed, Orphanet)

Patient Empowerment & Social Awareness

Usually carried out in close cooperation with:

- * Patients' Associations
- * other NGO's and/or
- * Public Institutions

An area of special attention has been the identification and removal of educational hurdles for children with rare diseases

A pilot observational+interventional program identified the major factors impacting educational access and integration of children with rare diseases, and how these could be approached and improved. Methods, findings & recommendations were later published as a book.

Another, still ongoing initiative fosters wider and better awareness and support among their teachers and classmates. Progress was presented and discussed at the 1st International School Congress (Jan '14), solemnly opened by HRH (now Queen) Letizia

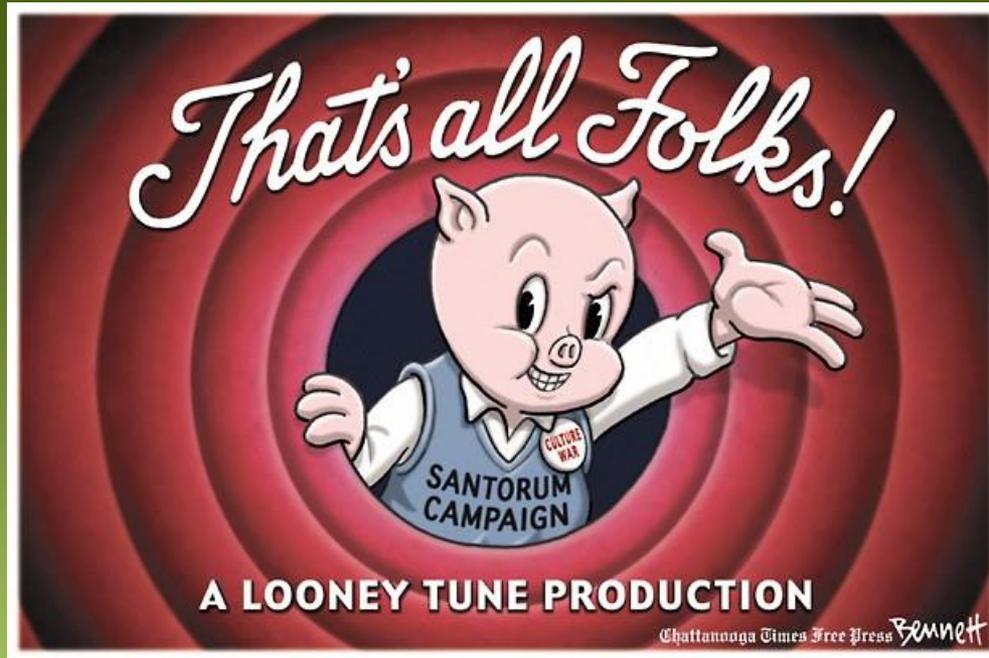
Other, cross-sectional initiatives



As a recent example, we found the book **“Rare Diseases: Challenges and Opportunities for Social Entrepreneurs”**, edited in 2013 by Nick Sireau, extremely interesting and useful for many Rare Diseases stakeholders. Unfortunately, not so many people in Spain know English well enough to read it comfortably.

Thus, we got the rights for translation and free-of-charge distribution of a Spanish edition.

Earlier this year, over 1000 printed copies were jointly distributed w/ FEDER (Spanish Federation of RD Patients Associations) to key stakeholders: National & Regional Healthcare, Education, Public Finances and R&D Authorities/Policy-makers; Scientific, Medical & Pharmaceutical Societies; RD working/special interest groups; unions & trade associations, media, etc. It was very well received and appreciated. Furthermore, it’s also freely available for download (as pdf) at our website



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Thank you for your attention!