

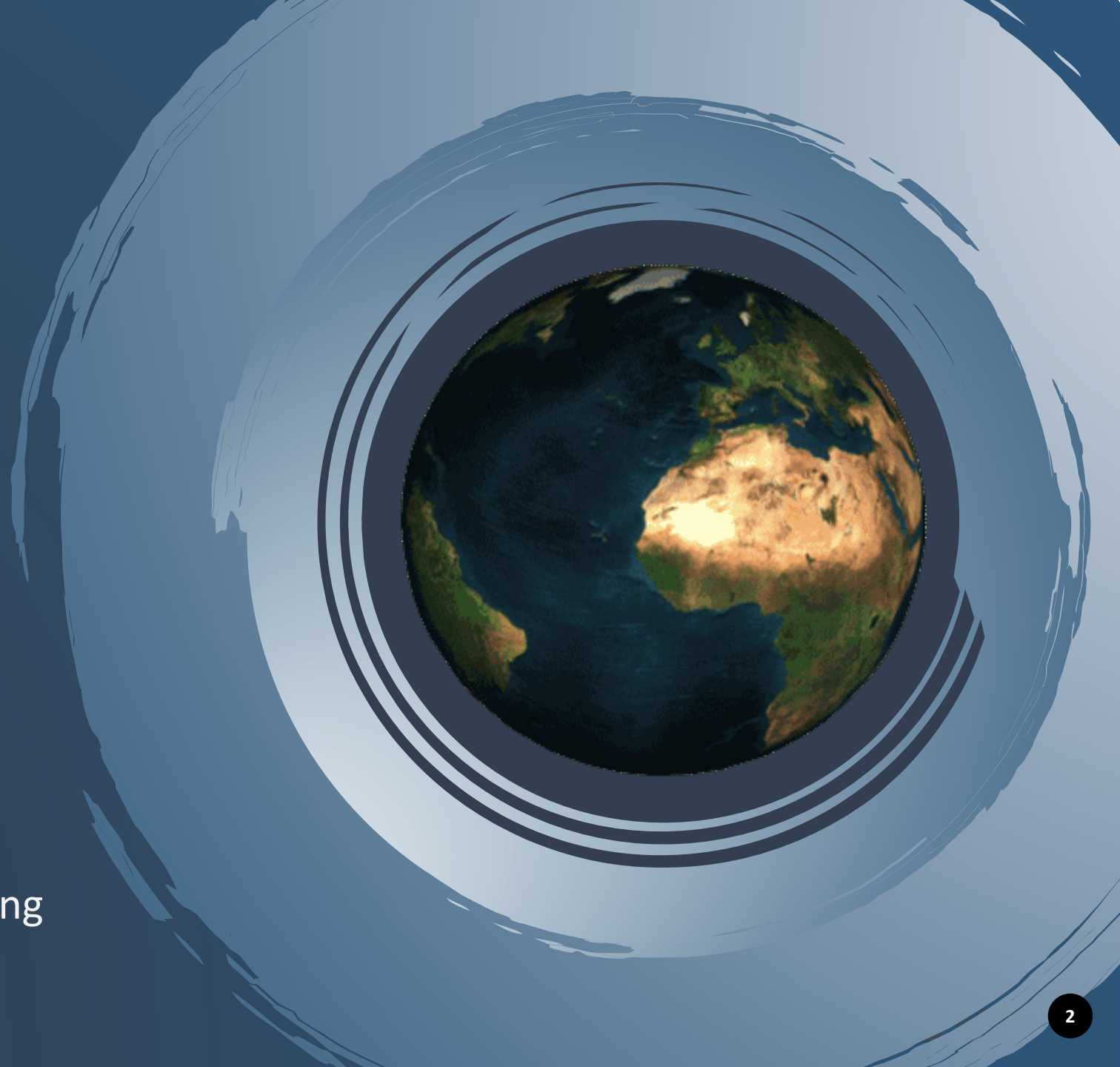


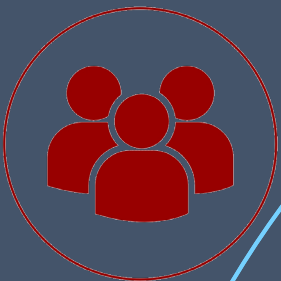
**EUROCAB,
EURORDIS 'S
programme for
COMMUNITY
ADVISORY BOARDS**

A NEW PERIOD

THE MOST AMBITIOUS PATIENT ENGAGEMENT PROGRAMME IN 2021

IN EUROPE, AND PROBABLY BEYOND
Framework is in place, with guidance,
principles, tools, templates, and training





8+ Active CABs created

- Cystic Fibrosis
- Limb Girdle Muscular Dystrophy
- Psoriasis
- Dravet syndrome
- Hered. Haemorr. Telangiectasy
- Multiple Sclerosis
- Head and Neck Cancer
- Myelodysplasia



And also (autonomous)
Cystinosis, Duchenne Muscular
Dystrophy, Angelman, Lymphomas,
Thalassemia...



21 Charters signed with industry
(Good Engagement Practices)



43 products discussed



102 CAB trained members



36 clinical trial
protocols
reviewed



Training

62 hrs online

100 hours f2f



22 companies involved

20 CAB meetings over a **4** year period

- Fruytier SE., Eva Vat L., Camp R., Houÿez F., De Keyser H., Dunne D., Marchi D., McKeaveney L., Pitt R., Vaughn M., Zhuravleva E., Schuitmaker-Warnaar TJ.
- “Monitoring and Evaluation of Patient Engagement in Health Product Research and Development: Co-creating a Framework for Community Advisory Boards.”
- J Patient Cent Res Rev. *In press*
- Co-authors include 4 patients’ representatives, 5 sponsors’ representatives and 3 academics



Academic excellence

Journal of Patient-Centered
Research and Reviews
In press (January 2022)

SUCCESSES

- Collection of long-term impact to demonstrate value

- Engaging with the EMA

CF-CAB shared its concerns on a new product with chair of CHMP (unsolicited opinion)

EURORDIS helped with content

CHMP chair responded in 3 days

Both documents = precious evidence

- Solving problems with complex trial design

Duchenne CAB advised Biophytis on how to improve a complex trial protocol for the MYODA trial, for the clinical development of BIO101

Company had been struggling to obtain IND status at FDA

CAB discussions helped convince FDA, and IND status obtained

- Solving problems with company's attitudes

Company X criticised the appointment of CAB members, and insisted to set the meeting agenda

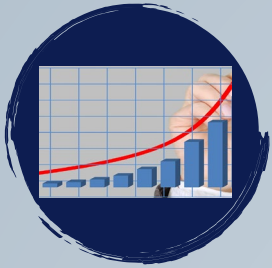
Wanted to control the CAB

EURORDIS intervened and explain the Good Patient Engagement Charter

Problem solved

Pilot is successful, yet need to re-organise the programme

And partner with a patient organisation: EUPATI Spain



Budget

Difficulties to balance budget over the years

Some federations have very small budgets (need to lend funds)

Complex funding mechanism, not as transparent as desired

Decision made not to collect funds directly from industry (33% cap)



Scaling-up

More than 500 products in R&D for rare diseases

Need to hire more staff to mentor the CABs

Otherwise risk to create a bottleneck

Yet, mentoring is critical for success

Other roles include administration, prospect, communication...

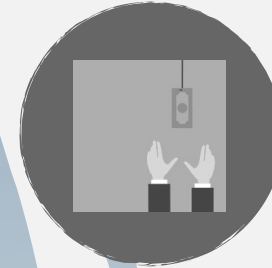


Compliance issues

Industry needs to declare funds they give to patient groups

For EuroCAB, EURORDIS is funded via the CABs (who invoice the sponsors they meet with). Uncertainties on how much to declare (Sunshine Act)

More transparent if sponsors could pay EURORDIS directly



Competition

Consultants, patient-driven or not, propose to facilitate interactions between industry and patient groups

But at a much higher price compared to EuroCAB practices

Concerns about VAT

Some EURORDIS members already engaged with EURORDIS competitors



Structure

Programme best hosted by a structure that can receive funds from industry

And with administrative skills (invoices, contracts, confidentiality undertaking, inside trading prevention etc.)

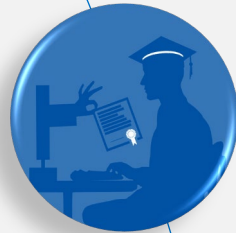
EUPATI Spain



A partnership agreement is now signed: EUPATI Spain is 100% responsible for EuroCAB management. Their costs should remain close to EURORDIS costs



EUPATI Spain is a patient organisation, independent from EUPATI "central". One board member is chairing a CAB already. Rob Camp is president and will continue mentoring CABs



Main activity: training patient advocates on the R&D of pharmaceuticals



Ability to receive funds directly from sponsors. An advisory platform with EURORDIS and other partners to advise EUPATI Spain

EuroCAB to contribute to policy discussions

2022 a crucial year for patient engagement



International Conference for Harmonisation

Revision of E6 guidelines

Need to propose effective methods to engage patients with developers of medicines, and with regulators

(evidence based)



Council for International Organizations of Medical Sciences

WHO + UNESCO to adopt guidance

On Patient Involvement in the Development and Safe use of Medicines



Future Cooperation on HTA in Europe

Patient participation in HTA

EUnetHTA21 launched to develop methods, including how patients can contribute to joint consultations and joint assessments



**A HUGE THANK
YOU TO ROB
CAMP!**



QUESTIONS?

François Houyez, Director of Treatment Information and Access 

+331 56 53 52 18 

francois.houyez@eurordis.org 

Houyez F. High Price Medicines and Health Budgets: The Role Patients' and Consumers' Organisations Can Play 
European Journal of Health Law. 18 May 2020, Volume 27: Issue 3,309–323.
DOI: <https://doi.org/10.1163/15718093-BJA10008>