# EXPERIENCES WITH CABS AND IDEAS ON IMPLEMENTATION

## Patients setting the agenda

Rob Camp

27 October 2017, Paris

EURORDIS.ORG

# Contents

History

Historical Examples

Historical Accomplishments

Two current examples

Clinical Trials Charter

Memorandum of Understanding

Eurordis CABs today and tomorrow

# Community Advisory Boards

Patients meeting with the decision makers of research and development organizations, including the pharma industry

Transparency

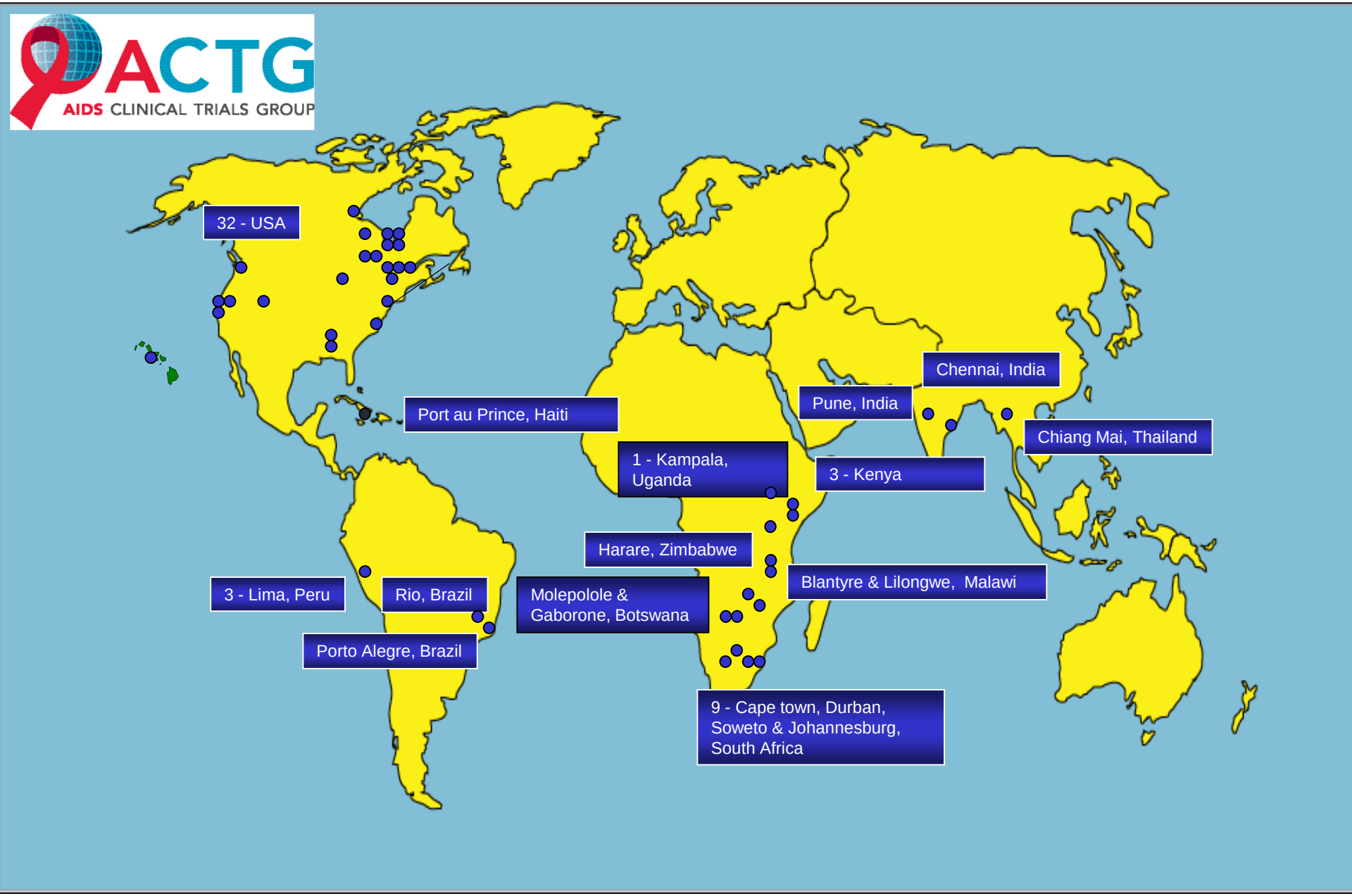
Conflict of Interest

Long-term committment


STOP AIDS
DROP DEBT

# Community Participation in Research?

The mission for community involvement is to provide meaningful and broad input into the scientific efforts, operations, and activities of the Research (network, company, sponsor).


ACTG
AIDS CLINICAL TRIALS GROUP
32 - USA
Port au Prince, Haiti
1 - Kampala, Uganda
Chennai, India
Pune, India
Chiang Mai, Thailand
3 - Kenya
Harare, Zimbabwe
Blantyre & Lilongwe, Malawi
Molepolole & Gaborone, Botswana
3 - Lima, Peru
Rio, Brazil
Porto Alegre, Brazil
9 - Cape town, Durban, Soweto & Johannesburg, South Africa

# CAB collective activities are focused on

Ensuring that ACTG **scientific priorities reflect the pressing needs of people living with HIV/AIDS**

Protecting the **interests of research volunteers**

Representing the interests of the diverse communities impacted by HIV

Advocating for as **broad inclusion** as appropriate into all ACTG clinical trials

Seeking innovative solutions to facilitate inclusion of traditionally **under-represented populations** within our studies

Making the **process** of designing and conducting studies **more transparent** to help preempt fears of collusion

# ACTG contentious

"I remember in 1990, I was heading to an AIDS conference in San Francisco and playwright Larry Kramer was being interviewed by National Public Radio," Hirsch recalls. "Kramer said no investigators were listening to the community and he suggested that maybe an assassination would get our attention. I was very scared and hid my name badge under my jacket the entire meeting."


FULLY FUND
THE GLOBAL
MONEY FOR
SAFER SEX
SILENCE=DEATH
WORLD
SAYS
NO TO
BUSHI

NIAID Director Fauci took charge of the situation suggesting that community members be allowed to attend the ACTG's meetings. By the end of 1990, each site sought the local community's involvement when developing studies.


New York Post: David Rentas

**GAY RAGE:** *Demonstrators mass outside Health Dept. offices in lower Manhattan yesterday to protest a city decision to cut in half its estimate of the number of AIDS-infected New Yorkers. Four were arrested as protest leaders called the city decision a maneuver to cut health services to AIDS victims.*

# Investigations and community

"One of my most vivid memories with the ACTG is when one of the activists came up to me during the first ACTG meeting in Washington, D.C. that included community members," Phair says. "He said he was discouraged because he was convinced we had some treatment we were withholding. After attending our scientific sessions, he realized there was no such magic treatment and that this was just going to take a lot of hard work. Dr. Fauci had been correct. Including the community was necessary to diffusing these myths about research."

# The AIDS Clinical Trials Group (ACTG) Global Community Advisory Board Committee Overview

There has been community representation in the network since **1990**. The GCAB was formed in March of 2009, as a new community structure within the ACTG, to provide a forum for community interests in the ACTG. The mission of the Global Community Advisory Board of the ACTG is to integrate community involvement in the AIDS Clinical Research Sites (CRSs) in order to advance HIV/AIDS research.

The GCAB is comprised of a member from each ACTG CAB and thus represents the community of the entire network. Issues discussed within the GCAB are **support of community outreach, education and participation in research**.

# The AIDS Clinical Trials Group Global Community Advisory Board Committee

GCAB provides an opportunity for affected communities, especially clinical trials participants to:

*Understand the clinical research process;*

Voice concerns regarding specific **clinical studies, their development, implementation and outcomes;**

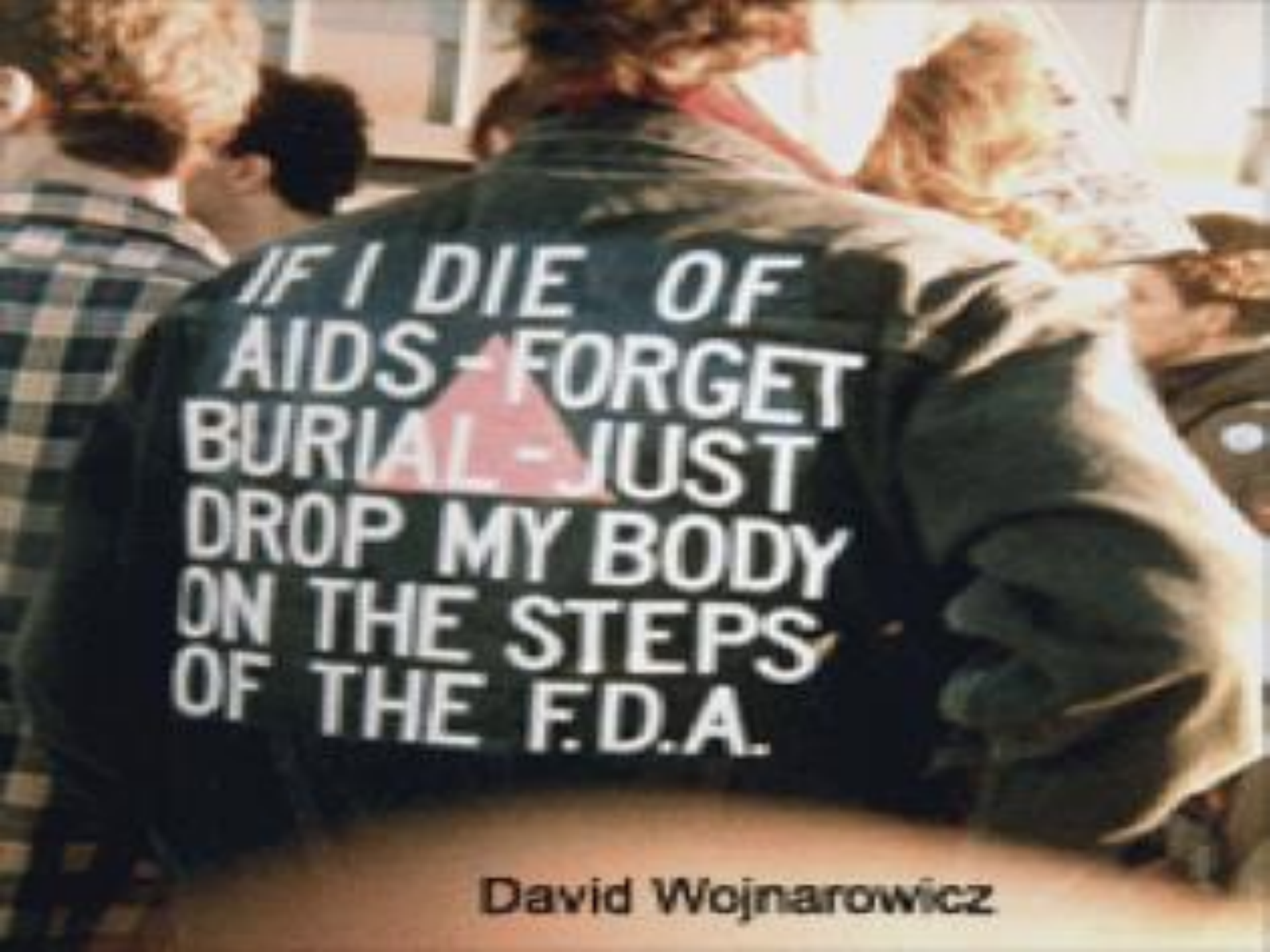
Give assistance concerning issues related to the accrual and retention of trial participants;

Give clinical trial participants necessary **advocacy**;

Forge **a viable partnership** that will lead to improved knowledge of HIV/AIDS disease;

Give a means to address grievance issues; and

**Promote ethical research purposes and practices**.




David Wojnarowicz

# ECAB

**Reviewing clinical trial protocols giving the point of view of patient community**

Promoting best practices, procedures and ethics

Promoting universal access to fair, sustainable, affordable drugs

Promoting **research developments that improve the quality of life** for people living with the disease

# ECAB

The European Community Advisory Board – ECAB was created in 1997. At the time, **patient advisory boards only existed on an ad-hoc basis and were convened by the pharmaceutical companies**, a major limitation that ECAB successfully overcame by putting forward an innovative model for the patient community to provide meaningful, independent, and valued input in HIV treatment and prevention research.

ECAB is a high-level scientific platform that brings together **expert patients and treatment advocates, scientific researchers, the pharmaceutical industry and international institutions** to address **key science and policy issues** related to HIV and its main co-infections, like hepatitis C or tuberculosis.

# Results with 1 sponsor

MEAT (multi-experimental agents trial)

GRACE (Gender and Race)

more reflective of the epidemic, now standard

Entry criteria

Drug users; high ALTs/ASTs

Informed Consent

More comprehensible

5 drugs approved in 9 years (HIV, HCV, TB)

More on the way (microbicide, long-lasting injections)

# New things today

# Today

CURE: Broadly neutralising antibodies (bNAbs)

- Cow antibodies (even-toed ungulates) - microbiome
- CAR-T (chimeric antigens)

Long-lasting treatments (once/month or less)

Treatments are prevention

Implementation of safe and effective prevention

- We fairly failed with condoms, same « mistakes » with PrEP?

# Why do this?

In HIV, in 1991 there were 3 drugs, all of which were so harmful that two of them were later discontinued due to toxicity and 1 was pulled back to third-line in a couple years.

Today, there are more than 30 drugs in 5 classes that have revolutionised treatment, care and the future for people living with HIV because from the time of those 3 drugs patients and their reps worked with/demanded better treatment and care.

# Treatment Activism-Advocacy

**Community must have representation** in government, the pharmaceutical industry & research institutions

Importance of **treatment education and mentoring**

Understanding (barriers to) **treatment development** and the regulatory process

Development of **relationships w/ other stakeholders** - companies, regulators, investigators, larger community

Continuing **education and trainings**

**Strategy development**-*pro-action instead of reaction*

# Treatment Activism – job description

Act as consumer watchdogs/community consultants to ensure ethical and equitable research and accessibility

Work with and Monitor sponsors (pharma, research entities, regulatory bodies), scientists, public relations staff, government, the media, politicians

# Do we need to do all this?

"Community" has to have a role in therapy development and knowledge production. If we don't define and take charge of that role, it will be decided for us.

- there are pressing needs of people living with diseases;
- research volunteers need to be protected;
- even the word "community" is complex;
- advocating for specific things in trials (extended clinic hours);
- helping to find innovative solutions;
- making design & conduct of trials more transparent

# e-Tuberous Sclerosis Complex

2010 – 2014 worked with Novartis on 3 trials of **everolimus**

Everyone signed the Clinical Charter and the MoU

Helped patient community consolidate (by-laws, statutes of European Fed)

worked on a patient registry

clear follow-up notes for next steps, etc

patient reporting

*Nothing happened. Novartis didn't heed advice for trials, never recruited*

Patients still willing to negotiate (at least 5 other companies…)

# Cystic Fibrosis Europe

*In one year:*

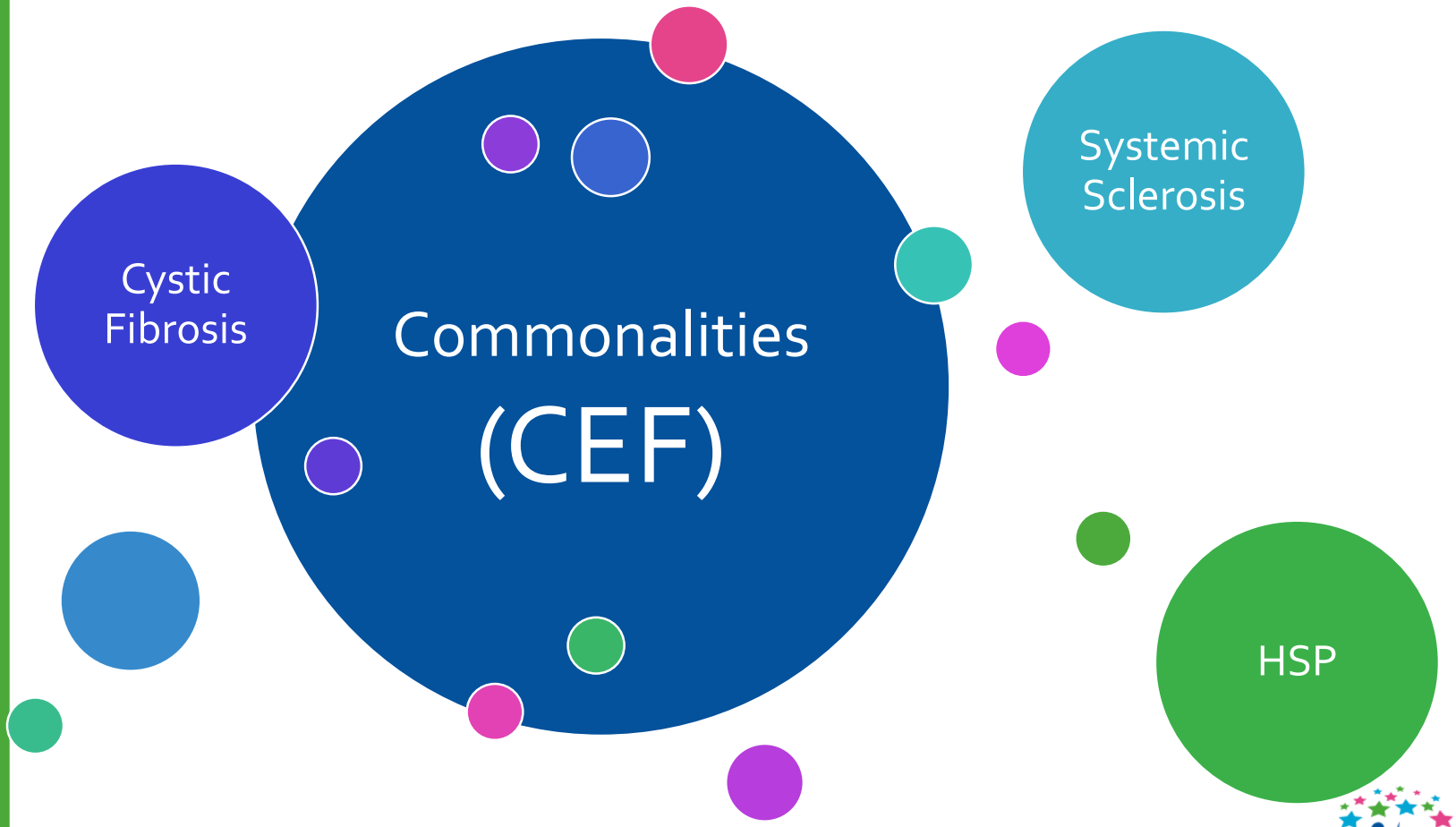
Set up meeting for whole process just one year ago.

- Two CAB meetings (with pharma)
- Two preparatory meetings

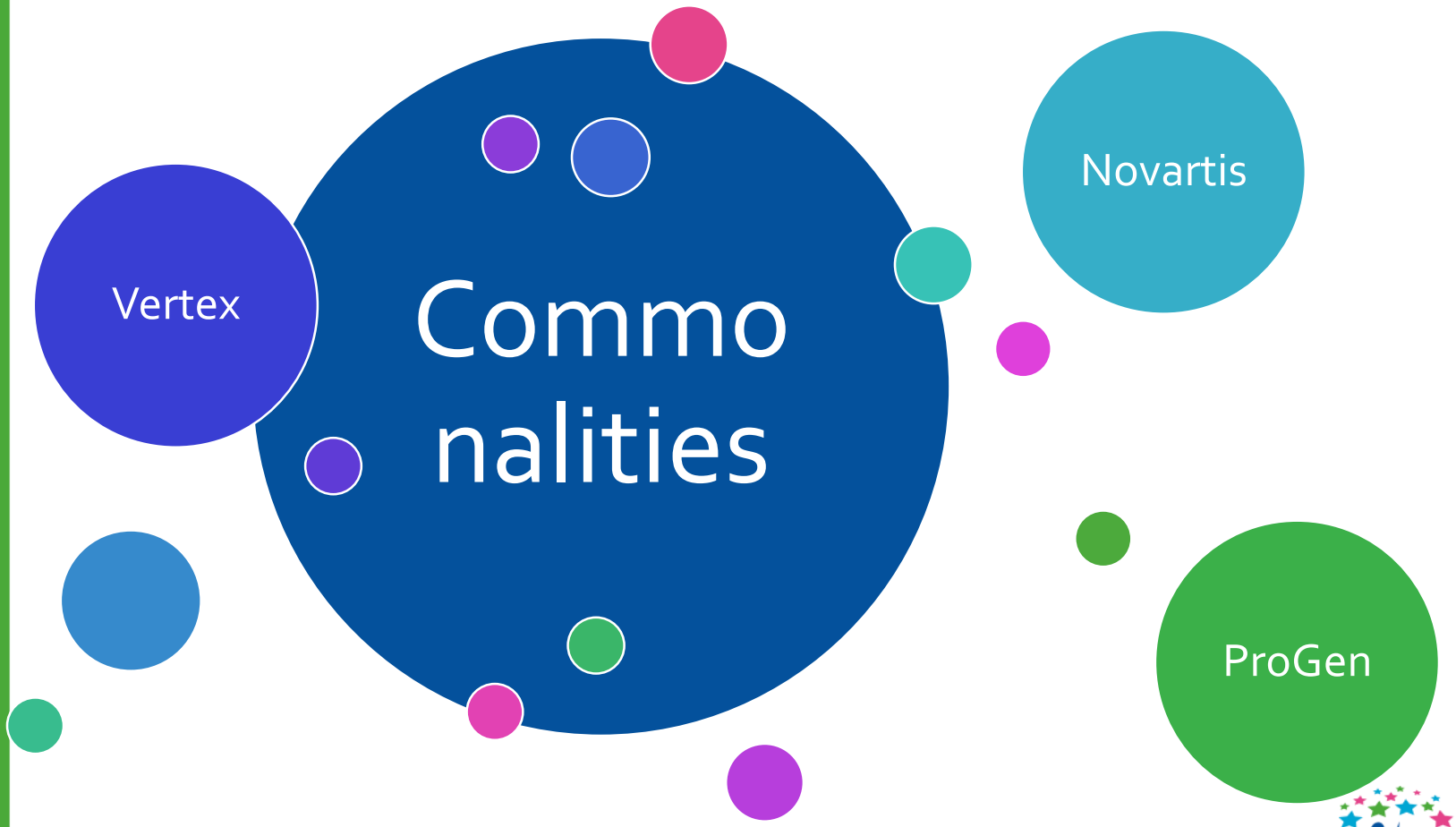
Success due to:

- Strong administrative core
- Active participation of members

# CABs


Cystic Fibrosis
Commonalities (CEF)
Systemic Sclerosis
HSP

# CABs



# CFE Zurich

**Wednesday** – half-day internal

introduction of ourselves, Review last meeting, QoL, (4) Central pillars – QoL, adherence, early intervention, Informed Consents

**Thursday** – all day meeting with Vertex (+ pre- and post-)

**Friday** – all-day meeting with Novartis (+ pre- and post-)

**Saturday** – half-day internal

Informed Consents, Structure, Eurordis listens in, Next steps

# Clinical Trials Charter

Sponsors and Patient Organisations (POs) share common objectives: **production and sharing of high-quality knowledge on diseases and development of safe and effective treatments.**

POs can collaborate with sponsors in all aspects of clinical trials:

- Adapting the design of the study to **patients' expectations, needs and realities**
- Providing **(early) information** to potential participants
- Supporting patients during the study
  - (confidentiality)
- Taking **Quality of Life** into consideration
- Discussing trial results **in a timely manner**

# General Principles

*Patient organisations (POs) should be informed of all aspects of the clinical protocol before collaboration.*

*POs should actively contribute to the documents aimed at patients - patient information document and the informed consent form.*

*Areas of and extent of collaboration could be enumerated in the "Agreement of Understanding", available to all stakeholders: patients, sponsors, investigators, ethics committees and national competent authorities.*

*Financial relationships (ie, between Sponsors and POs) are transparent.*

# General Principles

*Study results should be published, even in case of negative outcomes, non-conclusive or otherwise abandoned clinical trials.*

*Data acquired during clinical trials should be made available to the scientific community, with a view to fostering scientific progress and avoiding unethical duplication of clinical trials.*

*The commitment of a PO in the design and/or development of a trial does not modify the role and responsibilities of the sponsor, even if the study is financially supported by the PO.*

EURORDIS
Rare Diseases Europe

# AGREEMENT OF UNDERSTANDING

## FESCA & SSC and BI

# MEMO OF UNDERSTANDING

- Collaboration in Systemic Sclerosis and nintedanib

  - To refine objectives, improve the quality of results, improve the well-being of participants, as well as to accelerate the clinical research process, BI and FESCA/SSC have decided to engage in a transparent collaboration for Study 1199.214. In accordance with the *"Eurordis Charter for Collaboration between Sponsors and Patient Organisations for Clinical Trials in Rare Diseases"*, the scope and conditions of this collaboration are described in this Agreement of Understanding (AoU).
  - This AoU is a non-confidential and non-legally binding document, aimed to guarantee transparency in collaboration between BI and FESCA/SSC. It is available from both partners, including on their websites and on the EURORDIS website.
  - This AoU will be attached to all documents that mention this collaboration (fund-raising, submission to ethics committee, patient information leaflet,..).

# MEMO OF UNDERSTANDING

- A/ Initiators of the research project
  - This project was submitted by BI to FESCA/SSC

# MEMO OF UNDERSTANDING

- B/ Protocol design
  - Discussion between FESCA/SSC and BI covers
    - Information available to patients and when
    - Post-trial access to medication for patients

# MEMO OF UNDERSTANDING

- C/ Implementation of the Research Study
  - The collaboration between FESCA/SSCand BI covers
    - the study announcement
    - writing **patient information** documents
    - writing/editing the **Informed Consent** form
    - Informally talking to physicians about the study
    - **Videos** (for BI)
    - **Written interviews** (for BI)
    - **Newsletters** (possibly bi-annual) with articles on interviews with investigators, interviews with patients, devices used

# MEMO OF UNDERSTANDING

- D/ Conduct of the Research Study

  - The collaboration between FESCA/SSC and BI covers
    - The role of FESCA/SSC as an alternative source of support to participants during the conduct of the study
    - management of adverse events
    - discussion of possible interim analyses

# MEMO OF UNDERSTANDING

- Analysis and Dissemination of results

  - The collaboration between FESCA/SSC and BI covers

    - The analysis of results
    - the evaluation of possible benefits, including those based on secondary endpoints and Quality of Life criteria
    - Advice on writing scientific papers (for understandability)
    - dissemination of results to the patient community and general public
    - Thank you letters including approximate timing of results to be shared
    - Clinical trial lay summaries (advice and review)

# MEMO OF UNDERSTANDING

- Financial aspects and commitments

  - The study is not financially supported by FESCA/SSC
  - BI will cite this collaboration for the implementation of the study
  - BI provides financial support to FESCA/SSC for
    - The publication of a leaflet for the dissemination of results, even if they are negative (amount)
    - Announcement of results in FESCA/SSC's newsletters, etc (amount)

# Commitments…

- FESCA/SSC will announce the study in its Journal, on its website, with reference to this AoU
- FESCA/SSC will support the participants (members or not of FESCA/SSC) during the study
- FESCA/SSC will contribute to the lay dissemination of the results of the study even in case of negative results

- Future/ need a Mentor/Admin, they want to make agenda, call meetings, they can find 11 people, want to get in at an earlier stage (than Ph III), how to negotiate better

# MEMO OF UNDERSTANDING

- Other aspects of the collaboration not specifically related to the nintedanib trial
  - Disease awareness and education
    - Distribution of awareness cards ("So Rare" cards of SSc symptoms developed by FESCA) through BI
    - Awareness days and campaigns (traditional and social media)
  - Surveys and epidemiological studies (possibly not study-related)
  - Early dialogue around patient access to medicines/HTA and healthcare services
  - Health policy topics (post-study)

# CABs / Charter for Clinical Trials

A service to Eurordis members

Based on their needs and capacities

**Guidelines** and advice for all

**Mentor** to help either administratively, educationally or both

Eurordis can **liaise** with the companies

Not a service to Industry (Industry can express interest)

# Expected outcomes

Trial quality will improve

Patient interest in research will grow

Chances of a positive outcome of the trial/development due to

- Better design, smarter comparator, patient-friendly practical aspects
- Patients are retained due to better info flow, better follow-up of SEs

Regulators and HTA can make better and faster decisions on QoL aspects, reimbursement

# Documents

- What is a Community Advisory Board?
- Standard Operating Procedure
- Why and how to create CABs?
- Disclosure of Interest
- Charter and MoU
- Code of Practice
- How to Apply

Partie 4

# Slide de transition

EURORDIS
RARE DISEASES EUROPE

# Lorem ipsum dolor sit amet adipiscing

- Lorem ipsum dolor sit amet, consectetur adipiscing elit. Phasellus ut mi in orci elementum iaculis. Aenean a nunc. Proin dapibus arcu. Donec cursus pharetra massa. Ut suscipit tortor sed ligula. Maecenas risus urna

- commodo id, aliquam sit amet, rutrum eget, est. Maecenas id neque eu mauris ullasque pulvinar dui id erat. Duis sed sapien vel odio lobortis adipiscing. Nullam a justo. Nam ut eros et roin dapibus arcu. Donec cursus pharetra massa. Ut suscipit tortor sed ligula. Maecenas risus urna

- commodo id, aliquam sit amet, rutrum eget, est. Maecenas id neque eu mauris ullasque pulvinar dui id era eros et orci commodo sollicitudin.

EURORDIS
RARE DISEASES EUROPE

# Thank you for your attention.


## Name

Job title

Tel: Phone number

Email address


EURORDIS.ORG