Community Advisory Boards in Rare Diseases





Can patients change the clinical study paradigm?

Rob Camp, Senior Programme Manager CAB Programme, EURORDIS; **François Houÿez**, Treatment Information and Access Director, Health Policy Advisor, EURORDIS. **Methodology:** CABs give input via face-to-face meetings as well as teleconferences on a wide variety of topics that they are the experts on: patient outreach and site selection, education on research, clinical studies and their design, criteria for participation, informed consent forms and processes, compassionate use programmes, retention of participants, and reporting on results. Input is also possible on other topics whether it be clinical endpoints and how they are measured, meaningful patient relevant outcomes (PROMs), disease registries and their common elements & other factors that are meaningful to patients when measuring health and social outcomes.

Introduction: Patient Community Advisory Boards (CABs) are groups established and operated by patient advocates. They facilitate discussions in a neutral setting on the latest developments and challenges related to medical research and procedures in their disease area with the company or sponsor conducting the research. By being involved before a clinical study starts, patients help ensure that clinical studies are designed to take into account their real needs, resulting in higher quality research. CABs can also be involved in policy-related issues like access. They provide expert advice to all stakeholders involved in the research, development and service provision of



Results: Throughout history, medical science has evolved with little to no input from the people being studied. Actually, the

medical treatments.

The EUROCABs programme will offer:

Unique Guidelines and Specific advice

The CHARTER

The Memorandum of Understanding

The Code of Practice

A Mentoring Programme

The EUROCAB website

Training and preparation (ie, clinical trials; negotiation skills)

Administration (budget, logistics)

The EUROCAB seal (quality assurance)

disease is being studied, where the patient is nothing more than the vessel! CABs change that, and help to design, carry out and communicate on studies that are more rational and inclusive from a patient's point of view. By doing so, a bond of trust between the patient and scientific community is formed. Since the '90s, CABs have operated in a number of disease areas and have contributed to the amelioration of HIV and the cure for hepatitis C, for example. We are looking forward to similarly successful results in rare diseases.



Discussion: The EUROCAB programme will give you: **Experience**: CABs benefit from experienced advice on how to operate a CAB and to do it right; **Training**: CABs benefit from EURORDIS training programmes (Summer School, Winter School);

The EUROCAB programme: incubator, mentor, advisor



Identification of areas where CABs are needed

Call to Members
Feedback from Committee for Orphan Medicinal Products, Priority Medicines, EMA Working Parties...
Webinars to patient networks, meetings, preparatory phase (3-6 months)



Matchmaking with industry

Contact or help contacting developers /sponsors that correspond to the needs
 Receives direct requests from developers



Preparing/help preparing and running CAB meetings Keeping guidelines up-to-date, developing policies, qualification with authorities Backend office, "treatment activist advice"

EURORDIS RARE DISEASES EUROPE

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For further information, please get in touch with rob.camp@eurordis.org or francois.houyez@eurordis.org

Credibility: CABs benefit from EURORDIS

credibility and strong governance; **Standard**: CABs may qualify for regulatory procedures as "patient investigators"; **Up-to-date**: CABs will be aware of all new initiatives along the products life-cycle; and **Visibility**: CABs become more widely known!

EUROCAB website coming soon.

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