SCIENTIFIC ADVICE: HOW WE SEE THE ROLE OF PATIENTS

Session 2 – Improving Multi-Stakeholder Early Dialogues and Scientific Advice

François Houÿez

Symposium on Improving Patients' Access to Rare Diseases Medicines
13 February 2019, Brussels

EURORDIS.ORG

Evidence generation : a continuum

Evaluation guidelines and/or Community Advisory Boards (CABs): what can be done?

= A pre-competitive advice

- Multi-developers and HTA
- Explore patient needs
- Instrument selection
- Clinical Outcomes databases…

Scientific Advice (iterative) and/or CABs +/- HTA: how to do it?

- Study design, comparator
- Protocol development
- Mixed Methods
- Analysis & modelling
- Compassionate use

Evaluation, HTA, pricing: What's the promise?

- Benefit/risk
- Clinical meaningfulness
- Relative efficacy
- Uncertainties
- Utilities
- Reasonable price

More Evidence

Preclinical / Pre IND | Phase I | Phase II | Phase III | MAA HTA | HTA P & R | Post-Marketing Studies

Endpoint Strategy

Methodology Development

Evidence Generation and Analysis

Post-launch studies (PAES, PASS, PLEG): Is the promise confirmed?

- Pharmacovigilance+++
- Real-life benefits
- Patient satisfaction, adherence
- (Market entry agreements)
- (Observational studies, registries)

EURORDIS RARE DISEASES EUROPE

Steps

Not enough time!

Intimidating!

Frustrating!

Find patients

Agency own database and EURORDIS when OMP

Mentor

By agency and/or EURORDIS

Explain procedure and role

DofI (+ gvt and health insurers)

Confidentiality undertaking

Documents (e-meetings)

Involve

Not only to respond to questions, but elaborate their own

In all preparatory discussions

In face-to-face meeting (accompany them if needed)

In feedback

Evaluate input

Questionnaire to developer

Questionnaire to experts

Questionnaire to patients:

- Do you think your opinion was listened to?

- If not, explain

- Did your advice differ from the one expressed?

Acknowledge input

No name disclosure

Name organisation and/or country

EURORDIS RARE DISEASES EUROPE

D -120	• Letter requesting Scientific Advice / Early Dialogue
D -100	• (when relevant), contact EURORDIS to identify patients (share SA request letter) and/or own database of experts
D -75	• **Teleconference** with developer (clarifications and first questions) – **patients included. Ask patients which questions they have**
D -60	• Responses from developer (in writing, **shared with patients**): final documents
D -15	• **E-meeting** between experts and **patients**, key issues discussed, including patients' issues
D 0	• **Meeting**. Developer can also invite **patients** (e.g. CAB members), or CAB letter
D +15	• Written answer, with the **views of patients and reviewed by patients**

Patients come in

EURORDIS RARE DISEASES EUROPE

4

Questions to you

- Do you think patients should always be invited and attend the face-to-face meeting? Or only when HTA decide?
- Do you think patients should receive the same materials than other experts, or only some of it? Could patients discuss issues with others?
- Do you think patients who never met with the developer should be involved? Or a mix of "naïve" and more "expert" patients?
- SA or ED is a snapshot, and rarely iterative. EURORDIS believes it is the start of a dialogue with the developer. What do you think?

EURORDIS
RARE DISEASES EUROPE

Thank you for your attention.

François Houÿez

Director of Treatment Information and Access

francois.houyez@eurordis.org

EURORDIS.ORG