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
Evidence generation: a continuum

**Phase I**
- Preclinical / Pre IND
- Endpoint Strategy

**Phase II**
- Methodology Development
- Evidence Generation and Analysis

**Phase III**
- MAA HTA
- HTA P & R
- Post-Marketing Studies

**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

**Post-launch studies (PAES, PASS, PLEG):** Is the promise confirmed?
- Pharmacovigilance+++ 
- Real-life benefits
- Patient satisfaction, adherence
- (Market entry agreements)
- (Observational studies, registries)
Steps

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)

Not enough time!

Intimidating!

Frustrating!

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
Name organisation and/or country
No name disclosure

Not enough time!

Intimidating!

Frustrating!
- Letter requesting Scientific Advice / Early Dialogue

- (when relevant), contact EURORDIS to identify patients (share SA request letter) and/or own database of experts

- **Teleconference** with developer (clarifications and first questions) – patients included. Ask patients which questions they have

- Responses from developer (in writing, shared with patients): final documents

- **E-meeting** between experts and patients, key issues discussed, including patients’ issues

- **Meeting**. Developer can also invite patients (e.g. CAB members), or CAB letter

- Written answer, with the views of patients and reviewed by patients
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?
Thank you for your attention.

François Houÿez

Director of Treatment Information and Access
francois.houyez@eurordis.org