EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Scientific advice/protocol assistance and patient representatives

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Patients/Consumers Working Party (PCWP) and Healthcare Professionals Working Group (HCP WG) joint meeting

Plan

What is scientific advice
Who gives it
Where/When/Why is advice give
How is it given

When/Why will patients be involved
What will patients be asked to contribute

What is scientific advice

Set the regulatory hurdle for companies on the scientific requirements for new drugs:

- how to make them;
- how to test them first in the tube and in experimental animals; and
- most importantly: how to test them in humans in clinical trials.

Who gives it

Scientific Advice Working Party
of the Committee for Human Medicinal Products

- 30 experts from national authorities and university departments and hospitals chosen by required expertise: e.g. oncology, cardiology, psychiatry, neurology;
- scientific and logistic support from EMA staff;
- networking many thousands of EU experts;
- Experts chosen depends on disease are;
- Clinicians/academics/regulators

Where/When/Why is advice given

Voluntary, not mandatory procedure:

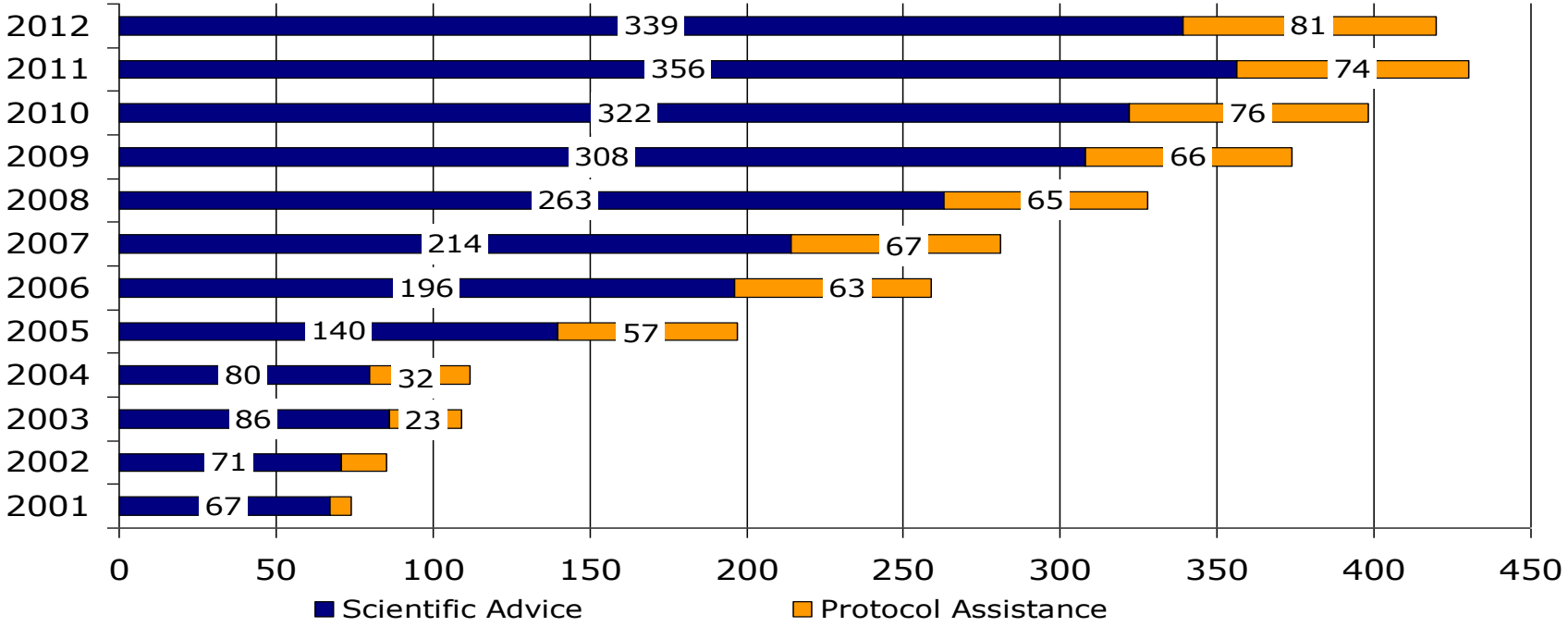
- companies ask questions though a scientific advice/protocol assistance procedure;
- responses are prepared and discussed by expert;

Example–question– Comparator Trials

In the sponsor's opinion a comparator trial or therapeutic use trial should not be conducted as part of the clinical development program for XXX Gel.

Does the Agency concur?

Scientific Advice main activity so far: product related scientific advice and protocol assistance for orphan drugs



How is it given

- The company submits a briefing document,
- in 50% of the cases, in particular when the regulatory experts do not agree with the company's proposal, a face-to-face meeting with the company is organised;
- written responses are adopted by the CHMP and send to the company: scientific advice letter;
- short procedure: 40 days or 70 days when a face-to-face meeting takes place.

When/Why will patients be involved

Rare diseases
currently we attempt to include patients representatives routinely but currently involvement in 1/3 approximately of procedures for rare disease

Rolling out to non-orphan diseases

Where patient's perspective will inform debate

When a face to face discussion meeting is planned at the EMA with a list of questions for the company

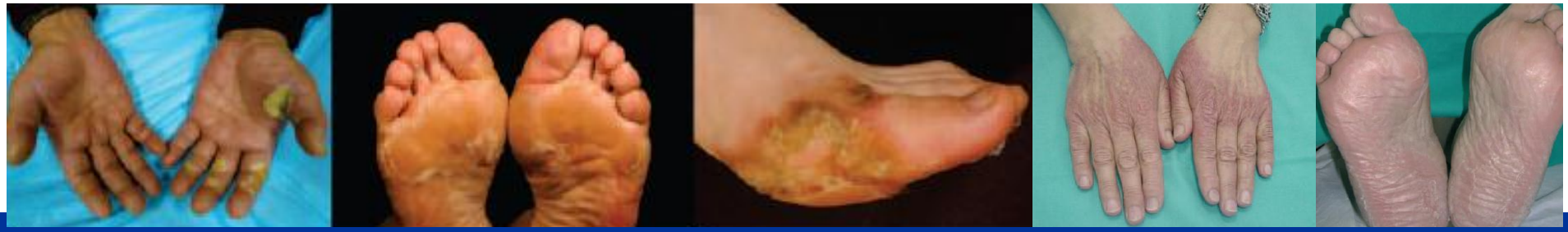
What will patients be asked to contribute

Not prescriptive / Depending on the issues but

To add real-life perspective

To add your views on the issues being discussed
e.g. feasibility or acceptability to patients of a proposed trial

<u>Unmet needs</u>
Types of restrictions in trial
How background treatments are being used in practice
Weight given to different outcomes for patients
Remind us why we are all here



Real life examples where patients involved

Clinical trials in non-healing wounds in epidermolysis bullosa

List of questions to company

"The proposed primary end-point (reduction of the wound surface area by 30%) is not in itself considered sufficient to demonstrate a clinically relevant benefit of the treatment and needs to be further elaborated on"

"The clinical relevance of a "temporary responder" is unclear and the clinical arguments to include temporary responders into the responder definition have to be improved"

Example 2 Topical treatment for ocular cystinosis

"The difficulties we had when she was young related to the administration of the drops, especially when at school as teachers were reluctant to take this responsibility .."

Who gives the drops?
Does this product provide longer lasting effect ?
Does it lead to an increase in eye irritation?
How long can this product last once vial is opened?
What kind of storage is required?

Endpoints; reduction of crystals, Photophobia, Visual acuity, Light/dark adaptation, corneal health, irritation and soreness, reduced number of applications per eye per day, ease of administration
increased compliance due to easier administration

Example 3 Enzyme replacement therapy in Mucopolysaccharidosis type IVA

The company is invited to discuss, based on phase 1/2 data and the natural history of the target condition,

a. the potential impact of limitations of the primary endpoint, highlighting the changes in rate of progression for 6 Minute walk test, 3 Minute stair climb test, and respiratory measures with or without XXX treatment

How will patients be involved

When a procedure is flagged for a rare disease / SA discussion meeting e.g. advice in late stage clinical trials/ new disease area/ new advice

Selected for patient representation (patient/carer/sibling/other)

Patients organisations will be asked for nominations; send us details

We will contact you – using normal, non technical language

How will patients be involved

We will send you documents for nomination as an expert if you are not in the expert database yet. Upon receipt of full original documentation, EMA will nominate you as an expert after evaluation of interests.

We check your declaration of interests for potential conflicts of interest.

We set up a Eudralink account for you – an easy way of sending confidential documents.

How will patients be involved

When all in order to proceed- we will send you in 1-2 emails - the background documents; a timetable of the PA procedure and approximate date of discussion meeting; additional scientific reports list of issues(questions).

Your comments can be optionally expressed in writing and we will give you a deadline; your comments will be circulated to the Working party.

We will inform Coordinators, patient organisation contact point and patients' representative on involvement of patients representatives.

We will send you an invitation for reimbursed participation the in discussion meeting through email/outlook (hotel, transportation).

How will patients be involved

Project manager will contact you directly (by phone- agreed time) to make sure that he/she understands his/her involvement in the procedure, and answer any questions you may have e.g. scientific issues.

During the meeting- time to express your views.

Afterwards in the plenary – also can express your views to the working party.

Opportunity to influence the outcome but is confidential.

After the end of the procedure, we send final advice letter, discussion meeting minutes, and acknowledgement letter signed by SA team leader with a feedback link to patients' representative and organisation contact point for information.

Other procedures

Parallel scientific advice with health technology assessment bodies

Now follows the rare disease process

Further information

Home EMA> Regulatory > Human medicines > Scientific advice and protocol assistance

We very much welcome patients additional involvement and look forward to seeing more patient representatives in scientific advice.

Questions?

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