EURORDIS
RARE DISEASES EUROPE

A NEW PILOT @ EMA

KNOW WHAT YOU EXPECT FROM A NEW MEDICINE

Patients' role in the R&D and evaluation of medicines in rare diseases

Discussions with developers

EuroCAB 2018- 2021
9+ active Community Advisory Boards
102 CAB trained members
36 products

Orphan drug designation

EMA COMP

Scientific Advice

EMA 2017:
Protocol Assistance: 33 patients
Scientific advice: 80 patients
With HTA: 18 patients

EUnetHTA 2016-2019
Early Dialogues
11 patients

Early dialogue on price

19 products,
12 patients

MOCA

Evaluation

EMA 2017
SAG: 46
CHMP
CAT

EunetHTA
Rapid REA
Patients involved for 12/14 HTAs

Coverage & Pricing

?

Timelines of medicine evaluation at EMA: Committee for Human Medicinal Products

Day 0

Marketing Authorisation Application submitted
2 rapporteurs appointed

NEW

≅ Day 70

Rapporteurs' first report
CHMP questions to applicant
Clock-stop

≅ Day 120

2nd report based on responses from applicant
Outstanding issues highlighted
Need for a Scientific Advisory Group meeting?

≅ Day 150

Scientific Advisory Group Meeting (SAG)
(# scientific advice meeting)
Doctors and patients respond to CHMP questions

≅ Day 180- 210

If major objections or outstanding issues: oral explanation with applicant, doctors and patients
Final CHMP opinion

+90 DAYS Authorisation to use the product in human published in the Official Journal of the EU + all EMA information published (EPAR, product information)

The process

For all submissions of a marketing authorisation application for a rare disease

When rapporteurs appointed

- EMA contacts EURORDIS with a template document
  - Product name, active substance name, disease / indication
  - Questions
  - Deadline (usually 3 weeks)

EURORDIS

- Identifies patients for phone / video conference interviews
  - Orphanet, ERNs, EURORDIS contact database
  - EMA template used as guide for semi-guided interviews
- Explores websites of relevant organisations to search for answers in relation to EMA questions
- Summarises responses in 2 to 3 pages

Memo

- Sent back to EMA / rapporteurs
- Shared with patients who were interviewed (usually 2 to 3)
- Sent to Marketing Authorisation Applicant (patients' names not mentioned)

Progress in 2021

27 interviews (1 hour each) for 8 products), and 3 failures / 12 (no report sent back to EMA)

Uplizna
(inebilizumab)
neuromyelitis optica spectrum disorders
6 interviews
February

Zynyz (retifanlimab) for squamous carcinoma of the anal canal
0 patients (ERNs, EATG…)
March

Sohonos
(palovarotene) for fibrodysplasia ossificans progressive
1 mother, 2 patients, 3 sites
July

Livtencity
(maribavir) for cytomegalovirus (CMV) infection
0 patients (ERNs, EATG…)
August

Vyvgart
(efgartigimod alpha) for generalised Myasthenia Gravis
3 patients, 1 site
September

Amvuttra (vutrisiran) for hereditary transthyretin-mediated amyloidosis
3 patients, 1 site
October

AUG SEP OCT NOV DEC JAN FEB MAR APR MAY JUN JUL AUG SEP OCT NOV DEC JAN FEB MAR APR MAY

20YY 20YY

Oxbryta (Voxelotor)
haemolytic anaemia in patients with sickle cell disease
4 patients and surveys
February

Omblastys
(omburtamab i-131) for neuroblastoma
16 groups contacted
2 patients and data from organisations' websites
June

Kinpeygo
(budesonide, micronized) for primary immunoglobulin A (IgA) nephropathy in adults
3 patients
July

Pyrukynd (mitapivat) intended for the treatment of pyruvate kinase deficiency
0 patients
August

Mycapsa (ocreotide) for acromegaly
3 patients, 1 site
September

Livmarli (maralixibat) Cholestatic liver disease in patients with Alagille syndrome
0 patients, 1 site
October

PILOT

- The rapporteurs can start analysing the data having in mind aspects that are important to you

• Know your unmet needs

Which treatment exist for your disease in which countries?

What benefits? What limits?

Highlight if there are differences between groups of patients about these aspects or if these views are generally similar across the condition

• Know the unknown about your disease

Any aspects about the condition or its treatments that you feel are not well-understood or not sufficiently considered

• Your expectations, relevant questions

What do you expect from a new treatment?

And from this one in particular (if you heard from trial participants or CAB discussions)?

Anything you feel is important for EMA to know?

CHMP pilot – early contact with patients

"In writing" procedure via a patient advocate experienced in the work of rapporteurs ("to put yourself in the mind of rapporteurs and of patients")

Interviews with patients extremely useful

To be considered for discussions on patient engagement in HTA

We should be doing it systematically and earlier in the process

CHMP / EMA are evaluating contributions received. Pilot to last at least one year. Other groups also involved.

You can prepare yourself and organise discussions with members, publish <u>your views</u>

Avoid copy-pasting information from HCP

THANK YOU

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