EUROPEAN SOCIETY OF CARDIOLOGY

RESPONSE TO THE EU CONSULTATION

"DELEGATED ACT ON POST-AUTHORISATION EFFICACY STUDIES"

The European Society of Cardiology (ESC)* welcomes the opportunity to comment on the consultation paper on "*Delegated Act on Post-Authorisation Efficacy Studies".*

The European Society of Cardiology (ESC) represents over 80,000 cardiology professionals across Europe and the Mediterranean. Its mission is "to reduce the burden of cardiovascular disease in Europe".

The ESC provides an array of scientific and educational activities, including the production and continuous updating of Clinical Practice Guidelines, the organisation of educational courses and initiatives, and pan-European surveys on specific disease areas. It also organises the ESC Congress, the largest medical meeting in Europe, as well as subspecialty congresses, in conjunction with its constituent bodies. The ESC edits and publishes 9 of the world's leading journals on cardiology.

THE CONTEXT OF A POST-AUTHORISATION EFFICACY STUDY

Consultation item No 1:

Do you think that a delegated act on the situations in which a post-authorisation efficacy study may be required will be of added value and that the Commission should consider bringing forward a draft delegated act? Please provide reasons for your opinion.

The European Society of Cardiology does not believe that a delegated act on the situations in which a post-authorisation efficacy study (hereafter, PAES) may be required will be of added value.

The PAES study should be decided individually depending on the development program.

EFFICACY VERSUS EFFECTIVENESS

Consultation item No 2:

Do you have any comments on the above? Do you agree that generally speaking post-authorisation efficacy studies should focus on generating efficacy data?

As effectiveness and reimbursement depend on individual Member States, the European Society of Cardiology believes that PAES should focus on generating efficacy data of real life patients. Nevertheless, pre-market access authorisation population should mimic as much as possible the

intended population. Professional organizations could help to identify appropriate "real life" populations for post-authorization studies.

SITUATIONS IN WHICH A POST-AUTHORISATION EFFICACY STUDY MAY BE REQUIRED

Consultation item No 3:

Please comment on the seven different situations described above. Do you agree that in these situations, a competent authority may ask for a postauthorisation efficacy study? Are there any other situations not covered by points 5.1 to 5.7 in which it would also be justified to oblige a marketing authorisation holder to conduct an efficacy study? If this is the case, could you please elaborate on these situations and, if possible, give specific examples to underpin the need?

In the cardiovascular field, PAES are not expected to be based on surrogates - unless these are well-known, correlate with clinical outcome and the product is a well-know class (e.g statins in lipid lowering). Otherwise, efficacy studies based on hard clinical endpoints should be conducted pre-market authorisation.

In the cardiovascular field, a PAES could be expected only to know long term efficacy data and possibly to conduct subgroup analysis in specific high risk patients such as the elderly or patients with diabetes.

STUDY DESIGN

Consultation item No 4:

Do you have any comments on the above?

The European Society of Cardiology agrees with the content related to the Study Design.

Depending on the product and the observed side effects in the pre-marketing studies, both randomized studies in specific subpopulations and registry-type studies in a population at large may be indicated.

AOB

Consultation item No 5:

Please feel free to raise any other issues or make any comments which have not been addressed in the consultation items above.

PAES can be initiated by the manufacturer or other bodies involved in health care when required, but should not be the sole responsibility of industry. They should be coordinated by regulatory agencies or HTA bodies, with the assistance of independent experts from professional scientific associations.