From: [redacted]
To: SANCO PHARMACEUTICALS D5
Subject: PCPAES/12/01 - Public consultation on PAES

Dear,

Hereby answers/comments from the RIZIV-INAMI Brussels to the public consultation on Post-authorisation efficacy studies (PAES).

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.

We support the Commission's effort to bring forward a draft delegated act on post-authorisation efficacy studies. In the Belgian Commission for Reimbursement of Pharmaceuticals (RIZIV-INAMI), these kind of studies are already requested and remain of interest in the future, because of the Belgian legal prerequisite to revise all reimbursement decisions of pharmaceuticals with added therapeutic value and of orphan drugs, within 3 years.

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?

These type of studies are particularly needed when marketing authorisation is granted based on soft end points and/or intermediary endpoints of efficacy. In our view, marketing authorisation granted on the basis of efficacy studies established by e.g. some enzyme levels, non-validated serum markers of diseases, or imaging of tumour without clinical relevance etc, makes it difficult to establish the relative therapeutic value of a new pharmaceutical. Hard endpoints on efficacy support much better the relative therapeutic value of the new pharmaceutical and hence, its financial value. For definitions on hard and soft endpoints, see EUnetHTA work package 5.

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?

The seven points cover indeed the situations involved here. As to situation n° 5.6, your explanation of long-term efficacy goes in the same line as developed by us in the previous item. We disagree that situations with a potential lack of efficacy in innovative therapies, are 'exceptional'. In our view, it occurs from time to time but it is not exceptional.

Consultation item No 4: Do you have any comments on the above?

It is clear that the design of the study should rule out biais as much as possible. We recommend in that perspective an article from The Lancet 2002;359:248-252: Bias and causal associations in observational research.

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.

No further comment. Your consultation paper is quite comprehensive and clear on this subject.

Best regards,

[redacted]

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