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RESPONSE TO THE PUBLIC CONSULTATION

At first I would like to thank you very much for this idea of public consultation regarding the complex problem of rare diseases.

In general I fully agree with all answers that you proposed in the consultation.

In particular in my opinion our country urgently needs prompt actions towards a management of orphan diseases. Among them (as in other European countries) creating a national programme for RD seems to be the most important nowadays.

In order to improve access to reliable diagnostic testing for RD, national reference diagnostic centres (clinical, laboratory and genetic) should be established. The cost of all necessary tests could be covered (at least partially) by EU budget.

Concerning equal access to orphan drugs, not only such hospital drugs, but also orphan drugs used chronically in the patients in ambulatory way, need to be funded at national level with strict monitoring by determined centres. The obligatory currently in Poland mechanism of the compassionate use of orphan drugs should be changed and simplified.

Moreover any suggestions for a care of adult patients affected by orphan diseases (as an increasing problem) could be added to the consultation.

Waiting for any recommendations from EC with regard to RD addressed to EU MS

Sincerely yours

Jolanta Sykut-Cegielska

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