ACHSE Response to IIA

Title:	Revision of the EU Legislation on medicines for children and rare diseases
Date	6.1.2021
From:	ACHSE – Rare Diseases Germany (Allianz Chronischer Seltener Erkrankungen (ACHSE) e.V.)
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The German National Alliance of Patient Organisations of People Living with a Rare Disease (*ACHSE – Rare Diseases Germany*) agrees with the analysis of the Inception Impact Assessment (IIA) of the current problems with availability and accessibility of medicines for rare diseases in Europe. Especially with the observation on page 1 that both legislative instruments have stimulated research and development of medicines to treat rare diseases and of medicines for children, while the legislation has not been able to stimulate development of medicines in areas of unmet needs (e.g. 95% of rare diseases still have no treatment option) or to ensure that European patients actually get the medicine(access), independently from where they live.

While ACHSE strongly supports the objectives of the IIA as summarized on page 3 and is willing to contribute to achieving those goals it wishes to stress at this point, that:

1. **Reducing or complicating rewards for the development of orphan drugs for rare diseases or even contemplating this** reduces the willingness of the pharmaceutical industry to invest capital in the development of OMPs in lieu of other investment options and therewith **deprives people living with a rare disease and unmet medical needs of future medication**.
2. **Reducing or improving those rewards will not have any influence on the actual access to authorized medication** and should not be presented as such. Any – justified - concerns on the financeability and sustainability of the European health care systems should be addressed heads on. **Indirectly reducing the development of medication in order to reduce costs should not be an option!**

German patients have enjoyed the success of the OD Regulations as Germany has given swift access and reimbursement of nearly all authorized Orphan Drugs. German patients have much to lose if the modifications of the EU Regulations hinder the development of much needed medication for the thousands of diseases for which there is no medication at all. They equally have much to lose, if the costs

for orphan drugs spiral out of control and the current reimbursement of all available drugs cannot be continued. Particularly, any split of common diseases into many subsets of supposedly rare diseases in an area, where there is no market failure should not be possible, and should – in our outstanding – already not be possible under the current regulations.

ACHSE would gladly contribute to the objectives of the commission:

1. To foster research and development of medicines for rare diseases and for children, especially in areas of unmet need and in better alignment with patient needs;
2. To contribute to ensuring the availability and timely access of patients to orphan and paediatric medicines;
3. To ensure that the legislation is fit to embrace technological and scientific advances;
4. To provide effective and efficient procedures, for assessment and authorisation of orphan and paediatric medicinal products

We strongly urge the Commission to broaden the analysis of the instruments the EU might have to foster improvements and achieve the above laudable goals.

Berlin, 6th of January 2021