Slovak Alliance of Rare Diseases,
Slovak Cystic Fibrosis Club,
Slovak Cystic Fibrosis Association,
Friends of salty children
(members of Eurordis)

The Standpoint of patient organizations in proceedings on drugs for cystic fibrosis number
32704, 32705, 32706, 32707, 32708, 32709, 32710, 32711
(expansion of indications for the causal treatment for the cystic fibrosis, correctors and modulators).

Whereas some issued standpoints in the above-mentioned proceedings **exclude the group of patients with a rare disease from possibility to access the treatment solely on the basis of a financial criterion,** posting such criterion to superiority as the only exclusion criterion for the decision on the expansion of the reimbursement of the treatment for the rare disease cystic fibrosis *(the so-called ICER/QALY financial criterion)*. Possible treatment access restrictions are introduced **directly as the recommendation not to expanse the treatment indications and indirectly by requiring the impossible to-be-fulfilled conditions when assessing the orphan medical product (OMP)**, this contradictory with the best practices and conditions applied within EU, worldwide and in the neighboring countries of our region.

Whereas rare diseases are defined in the **Regulation of the Council and Parliament No. 141/2000** as a life-threatening or chronic condition that affects in its prevalence less than 5 out of 10,000 inhabitants and this Regulation further defines that such a disease is so rare that it is necessary to create incentives and remove administrative obstacles so that these so-called ORPHAN medical products are created, marketed. The same philosophy has been applied by the USA since 1983 and Japan since 1993. The conditions for the development and entry of products for the treatment of a rare disease are to be set so that all patients have an **equal chance of access to treatment and the results of the progress of science and medicine regardless of the rarity of their disease.** This principle is enshrined in EU legislation **and the evaluation of OMPs solely on the basis of the requirement to demonstrate the cost-effectiveness is considered discriminatory against patients with a rare disease, as it prevents them from accessing the treatment**. Often, the OMP has no medical alternative and **covers an unmet medical need, which is also the case of this evaluated treatment and its expansion.**

Whereas the ICER/QALY criterion and the obligation to demonstrate the cost-effectiveness are not used in the common practice within the EU nor USA **for the assessment of orphan medical products**, as it a methodologically inappropriate criterion that poses an unfulfillable requirement on the OMP. The usual practice for evaluating the **OMPs** is the so-called **MCDA method** *(Multi Criteria Decision Analysis)* – decision making based on multi criteria analysis and assessment. The primary example from neighboring countries is the **Czech Republic, where the legislation in the identical area exclusively states that the cost-effectiveness criterion is presented as informative.**

Whereas the wording of the Slovak Act. No. 363/2011 Coll. on the reimbursements from the public health insurance **includes a separate route for the entry of orphan medical products**.

Whereas the financial criterion in the issued opinions is applied **disproportionately and unbalanced**, as meeting the threshold value does not mean the positive result on the expansion of the reimbursement, but failure to meet the cost-effectiveness criterion means the recommendation not to allow for the expansion of the indication. Such assessment puts the **patients at a disproportionate disadvantage**.

Since we believe that when assessing the treatment and OMPs for orphan diseases, in accordance with the EU policies, the foremost consideration shall be placed on **the severity of the disease, unmet medical need of the patient, demonstrated benefits improving patients life, therapeutical effectiveness and safety,**

recommendations of expert committees and organizations, including the patient`s organizations, the societal impact of the treatment, availability of the treatment in other EU countries and similar criteria.

As we are currently the only EU country denying the access to this causal treatment to some groups of patients with cystic fibrosis in line with the EMA approved indications and actually, we do have patients in critical condition falling under the indications in mentioned proceedings.

Since it is a **fundamental causal treatment** that, as per models, adds 35 plus years of quality life to patients in the 12+ cohort. According to demonstrated **initial studies** as well as **repeated studies after 5 years** of treatment and the clinical practice**,** the assessed treatment **reverses the progression of the disease.** And since the **clinical results** also already of our treated patients **prove the reversal of the progression of the disease in measured parameters and mainly demonstrate the decrease of the concentration of the chlorides in sweat to the level corresponding to a healthily person without cystic fibrosis.** This leads to a full return of our patients to society and life, when they do work, study, start businesses, even start own families, etc.

Whereas the treatment with **correctors and modulators** is the global standard in the treatment of cystic fibrosis and **it is included in our own valid standards of the Ministry of Health**.

Since the effect of the evaluated drugs is clearly and indisputably proven by standard studies accepted in all evaluation systems and in comparison with previous generations of causal and symptomatic treatment, **where even, these drugs were already evaluated as highly effective by our own HTA agency in the past proceedings**, as well as other evaluation organizations and systems within the EU.

Whereas the **disease knows no borders** and we have identical **genetical predispositions**, **prevalence** and we do believe that we shall have **and equal share that society spends on its patients, especially when comparing with other similar countries of our region** *(we repeat, we are the only EU country that currently denies its patients this treatment in certain indications approved by the EMA)*.

Therefore:

We please and ask all the participating entities for the MAXIMUM COOPERATION in acting, provided resources, data, information, coordination, negotiations and good will to come to the conclusion leading to availability of causal evaluated treatment for Slovak patients with cystic fibrosis at the level comparable with other EU countries, especially countries of our region.

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