Pracovná skupina pre
MODERNÚ LIEČBU CF
Working group for
NOVEL CF THERAPIES

Klub Cystickej Fibrózy

Opinion of the organisations of parents and patients with Cystic Fibrosis on the Proposal for listing the drug Orkambi to the list of categorized medicines and on the official agreement on its pricing

Cystic Fibrosis is a rare, genetic, incurable disease. It is a progressive and multi-organ disease. It affects, in a devastating manner, lungs, pancreas, liver, digestive tract, accompanied with diabetes, osteoporosis, infertility and nasal polyps. Current treatment of cystic fibrosis is only symptomatic, to slow the progression of the disease. At the same time, the treatment and live regime is extremely demanding in terms of time, physically, mentally and economically for the patients and their families, as well as demanding for the provided healthcare. Despite the great lifesaving effort from the side of the community and our healthcare professionals, we are losing our patients in a terrible way at a very young age, often before reaching adulthood. Cystic fibrosis does not affect intellect and patients in our community are able to achieve, despite the horrific diagnosis, and due to an extraordinary effort, self-denial and circumstances, amazing results in a variety of areas for benefits and inclusion into the society.

After many decades of research and development, staring in the 1950s to understanding the nature of the disease at the genetic and cellular level, there are breakthrough drugs marketed all over the world since 2012, called correctors and modulators, aimed to treat the root cause of cystic fibrosis; the failure of the CFTR protein. This treatment is revolutionary, the first and so far the only attacking the root cause and not only the symptoms.

In the communities of patients, having the access to these new therapies around the world, we do see amazing real-world improvements of the quality of life of patients *(significant prolongation of the life expectancy, significantly less inflammations, less use of oral and IV ATBs, less hospitalizations, increase of the lung functions indicators, delayed or unnecessary lungs transplantation, improved BMIs, taking less doses of enzymes, decrease of sugar levels, reduce insulin use...).*

The availability of correctors and modulators in countries around the world and in Europe increases rapidly, literally from month to month.

On behalf of the CF community, patients, parents and their families, healthcare professionals we want to ask and request to reach an agreement on pricing and listing this groundbreaking medicine into the reimbursement system; we do support the listing of the drug Orkambi into the categorization and reimbursement system.

No one is to blame for the fate that our patients and their families have taken, since there has been any treatment available until now. At the same time, however, we are convinced that as the development has reached the point when there is an effective root cause treatment available for the first time in the history, it is our duty, as a modern society in Europe, in a joint effort, to bring an equal treatment within the healthcare system also for the patients in Slovakia.

After many decades and generations of suffering patients, who could not get a chance to grow up and survive; there is for the first time a hope that the cystic fibrosis becomes a manageable disease due to existing treatment rather than a death sentence.

Thank you very much, Next page 2; Signatures

I agree and support the Opinion above

	Date	Signature
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