



Weekly Briefing

**Slovakia social briefing:
Fight against the SMA disease
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Fight against the SMA disease

Currently, there seem to be three topics most resonating in the Slovak society. The first one is the parliamentary elections that took place on 29 February, the results, possible future scenarios and challenges the new government will need to face. The second one is the globally spreading virus and measures being taken to combat it, as well as the need for solidarity and responsibility. The third theme, which has also been selected the topic of this social briefing, is the case of four little Slovak children with the SMA disease.

In the beginning of the year, the information concerning a baby boy Riško and his serious health issues spread through the Slovak media and social networks. Riško has been diagnosed with spinal muscular atrophy (SMA) – a genetic disease that affects muscle movement and the nervous system. There are several types of SMA; Riško suffers under the most serious one – type 1. The disease causes movement difficulties, disables swallowing and breathing and, in the worst-case scenario, can lead to death, which is likely to occur before the age of two. Usually, the symptoms are visible several months after a child's birth. Before, the disease was impossible to cure as there was no medication. Today, the cure does exist.

To fight the horrific disease, Riško needs a dose of medication, which is supposed to be the most expensive medicine in the world – it costs more than 2 million dollars. The problem is that the drug is not registered in Europe, meaning health insurance companies cannot cover the expenses connected with its purchase. Moreover, some Slovak health experts do not recommend it, as there is supposedly not enough scientific evidence that the cure really works and is harmless.

Moreover, Riško is not the only child in Slovakia with the described health issues. At the moment, there are three other children urgently needing the medicine. Except from the enormously high price for the dose, a crucial player in the game is time. Namely, the medicine needs to be taken no later than a child turns two.

In February, the parents of the four children were invited for a negotiation with the representatives of the Ministry of Health. The result of the meeting was not in favour of the families. Firstly, they need to cover the expenses linked to the treatment and medication purchase themselves. Secondly, it is not possible to administer the medicine in Slovakia, as it is not registered by the European Medicines Agency. The parents would have to find a doctor who would be willing to take the whole responsibility and provide the necessary treatment

linked to giving the dose. Medical practitioners in Slovakia usually do not have experience with this kind of treatment, hence it would probably be very difficult to find someone to carry it out.

Riško has been given several doses of another medication which is already registered in Europe. That one, however, does not cure the disease, it can only mitigate its progress. As that medication is registered in Slovakia, the expenses linked to the treatment have been covered by the health insurance.

One of the differences between the two kinds of medications lies in the way of their application. The cure registered in Europe – the one Riško has already been given – needs to be inserted directly into the spinal cord, and thus multiple times. On the contrary, the expensive medicine the parents of all four kids are trying to acquire, is inserted only one time into the vein. Hence, the application seems to be much less painful.

The medicine Riško and other three paediatric patients need is currently being evaluated by the European Medicines Agency. It is no easy process as many aspects have to be assessed, such as the effectiveness and safety of the medicine. Presumably, the evaluation process will not be completed sooner than in May 2020. Logically, the cure has to pass the evaluation on the European level so that the process can be initiated in Slovakia. That is expected to take approximately another six months. For some of the affected children, this would be too late.

SMA Europe has issued a press release concerning the evaluation of the medicine and the objective to make it accessible to European patients. *'Access of European patients to treatments will continue to be at the top of SMA Europe's agenda. We acknowledge there is still a long road to walk before European patients can access this new treatment. SMA Europe would regard it as unethical if the price would preclude any patient in any country from receiving this potentially life saving therapy.'*¹

After months of struggling on their own, parents of the little boy decided to ask people for help. A couple of months ago, a transparent bank account was set up to raise money for the necessary treatment for Riško. The parents were encouraged by past experience from Hungary, where good people managed to collect the money needed for two children suffering from the SMA disease.

The story of the little boy got viral immediately. Social media posts and news helped to spread the information through the whole Slovakia. Moreover, foreign media reported on the

¹ <https://www.sma-europe.eu/news/avaxis-receives-fda-approval-for-zolgensma-the-first-gene-therapy-for-paediatric-patients-with-sma/>

case of the baby boy. The rapid spreading of the information was triggered by several publicly known individuals, mainly sportsmen, who expressed their concern and let their voices be heard. One of them was Zdeno Chára, a legendary Slovak ice hockey player who has been the captain of Boston Bruins for several years now. As a matter of fact, Boston happens to be one of the places where the treatment can be provided.

In case of Riško, the seemingly impossible has become reality and the money for the cure has been raised. The parents have been constantly informing the public about the progress and expressing their frank gratitude. At the time of finalising of this briefing, Riško is getting ready to get his dose in Budapest. Now it's time to help the other three children, who also have transparent bank accounts where donations can be sent to.

The debate around the children was especially sensitive as it was happening simultaneously to the political campaign for the parliamentary elections that took place on 29 February. Several politicians – even the Ministry of Health and the Prime Minister Peter Pellegrini - are said to have offered help to the family of Riško – governmental aircraft in case of need for transport to Boston. However, the parents denied it arguing they did not want their son to become a means of political campaign. Besides, the parents are well aware there are many people in Slovakia urgently in need for expensive medications, and the state does not seem to be helping enough. It would simply not have been fair if they had accepted the help, they said.

The people of Slovakia have managed to raise the money needed for Riško's treatment. Some say, ordinary people have taken care about an issue which the state should be responsible for. At the moment of elaboration of this text, the parents of the other children with SMA sent an open letter to the President of the Slovak Republic Zuzana Čaputová. In the letter they are asking the President to request from the newly to be appointed Prime Minister and the government to allocate money for the treatment of their children. Their situation is getting complicated also because of the coronavirus, since it is weakening the children's immunity. The sooner the children will be given the dose, the more chances they have to live on. Nevertheless, the public is still helping, expressing solidarity, donating money and thus sharing even the little they have in order to help those who need it even more. One way to help is to send a text message to a phone number designed specifically for this purpose. We dare to say, without help and support of ordinary people, raising money for Riško would not have been a success.

It does not happen very often that problems of individuals, their destiny would attract attention of complete strangers. The situation concerning Riško and the three other children speaks in favour of humanity, solidarity and willingness to help without expecting something in return. Where the state is not able or willing to help, good ordinary people have taken over. Literally thousands of people contributed for Riško's treatment, as well as to other three children. Every single euro counts, so why not to share?

The question remains, how the soon to be formed government will react, whether they will find a way to help the other little patients. Otherwise, the three families will also have to rely on the help of ordinary citizens. We hope a solution will be found as soon as possible, as both the time factor as well as the threat of the coronavirus are making the whole situation much more complicated.