RZECZNIK PRAW PACJENTA

RZECZNIK PRAW DZIECKA

Mr. Alexander Hardy
CEO
BioMarin Pharmaceutical Inc.
105 Digital Drive
Novato, CA 94949

Dear Mr. Alexander Hardy,

we are addressing you as representatives of institutions established to protect the rights of young patients. In our actions we aim to ensure that every child – regardless of age, gender, nationality or severity of illness – has real access to medical services and feels safe throughout the entire therapeutic process.

We have recently received several hundred communications from children suffering from achondroplasia, or their caregivers. They point to the lack of access in Poland to the innovative vosoritide therapy with the [redacted] medicinal product.

Information from children with achondroplasia and their parents shows, that they struggle with a number of challenges in terms of health as well as difficulties of social and everyday life. The symptoms of achondroplasia that young patients suffer from are accompanied by physical pain associated with i.a. orthopaedic and neurological problems. The health problems of children with achondroplasia are well known to you. We would like to emphasise that children with achondroplasia are particularly vulnerable to the disorders in the sphere of mental health. These children struggle to accept the disease, they feel different from their peers, they experience stigma and rejection.

Currently, no drug technology is reimbursed in Poland for the treatment of achondroplasia. The only reimbursable surgery is the stretching of the radius, ulna, femur, tibia, and fibula. These procedures are associated with many possible complications, pain and periodic exclusion from social life, and only slightly do they alleviate some of the effects of achondroplasia.

In 2021 the European Medicines Agency has approved a modern and effective therapy for the treatment of achondroplasia with a drug called [redacted]. According to Polish medical experts involved in the treatment of short stature in children, vosoritide is the first and only drug technology for achondroplasia, and due to the serious and rare nature of the disease, the implementation of the therapy cannot be delayed.

In Poland, approximately 560 patients under 18 years of age suffer from achondroplasia. Taking into account the inclusion criteria for [redacted] therapy, the treatment affects around 220

young patients. [redacted] therapy would significantly improve the quality of life in this group of patients – not only in terms of health, but also in terms of social life.
We cannot stress enough how important it is to us that Polish children with achondroplasia feel safe and have the chance for a life of young patients with achondroplasia in other European countries.

The Polish drug reimbursement system is a system, in which the initiation of proceedings for systemic reimbursement for a given medicine, may only take place as a result of the submission of an application for reimbursement by the responsible entity or that entity's representative.

One of the goals of BioMarin Pharmaceutical Inc. is to improve the quality of life for people suffering from rare genetic diseases and the main motivation is the principle "Patients are the heart of everything we do". We are guided by the very same motivations. We therefore ask you to take the situation of Polish children into deep consideration and submit an application to the Polish Minister of Health for reimbursement of [redacted] - the only effective medicine for children suffering from achondroplasia.

We firmly believe that you will give these children the chance to grow up in peace and safety.

Sincerely yours,

Bartłomiej Chmielowiec
Ombudsperson for Patients

Monika Horna-Cieślak
Ombudsperson for Children