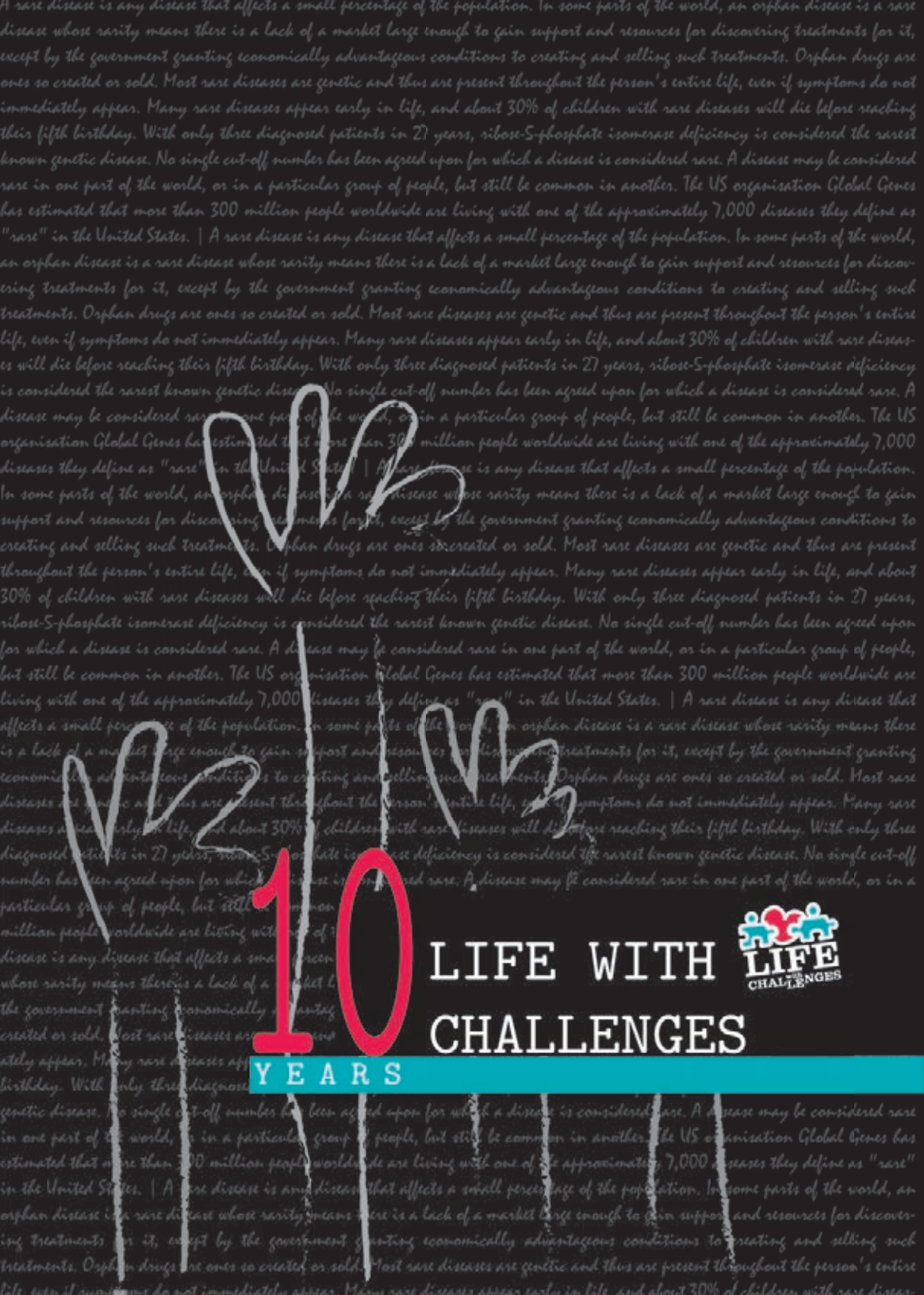
10
LIFE WITH CHALLENGES
YEARS

10 YEARS
LIFE WITH CHALLENGES

The membership in the association is voluntary and free for all patients and parents that face life with a rare disease. The association is also open to cooperation with medical professionals, researchers and supporters that want to advocate for better quality of life of families with rare diseases.

Additional information on LIFE WITH CHALLENGES:

- Web page: http://challenges.mk/
- YouTube: https://www.youtube.com/user/lifewithchallenges
- Facebook page: https://www.facebook.com/LifeWithChallengesi
- Twitter profile: https://twitter.com/ZivotPredizvici

LIFE WITH CHALLENGES is member of:

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I wish that researchers could invent therapy for all rare diseases, for some of them there is treatment, for others there is nothing. For me, for my disease Epidermolisis Bulosa, there is no treatment available, but I can live without therapy. I can be happy. I've got my mum and dad who are always here with me.

Iva Petrevska,
patient with rare disease Epidermolisis Bulosa

When I started this patient association, I thought that I am just a patient who can do so little. I was fighting for my life ... When more and more patients joined me, I felt how together we are stronger, and I knew we can change the world if we want to. I have met over 300 families facing more than 90 different diagnosis, each story is different, each suffering and pain are unique, but we had common challenges and problems that we decided to overcome together. Many times over the years I have felt desperate and helpless, thinking that hope will disappear ... the families have helped me to feel empowered and persistent in our requests to the institutions for better quality of life. Together we have accomplished a lot, more than I could have imagined, more than I thought it was possible.

10 years have passed, and there is still much more to be done. I am sure now that we can change things, because we are together, because we fight for a better future for all citizens of North Macedonia. We are rare, but we are not alone, we have each other. We are rare, but we are strong together. And we will always have hope and courage to do more, to live life to its fullest.

No one should lose hope, no one should stop fighting for a better life ... together we can accomplish the impossible. Together we will build future, we will make future happen for families facing life with rare diseases.

Vesna Aleksovska,
president of the association

Foundation of
LIFE WITH CHALLENGES

The Association of citizens for rare diseases LIFE WITH CHALLENGES Bitola, was founded with the help and support from the National alliance of people with rare diseases of Bulgaria. The first meeting of families and doctors related to rare diseases in North Macedonia was organized by the Bulgarian alliance in Ohrid, North Macedonia, in June, 2009.

Since then the patients in North Macedonia have decided to form their own organization and fight for their rights. This is how LIFE WITH CHALLENGES was born.

In the following years we were gathering members and learning how to manage and grow into a strong patient organization.

VISION

Our vision is achieving the best possible quality of life for patients and families with rare diseases, through best possible social and health services.

MISSION

Our mission is developing solutions and policies through implementation of activities for improving quality of life for patients and families with rare diseases. The goal of LIFE WITH CHALLENGES is to help and support

patients and families that face life with rare diseases. Our mission is realized through the following activities:

- Helping and informing people with rare diseases, creating and sustaining communication among patients
- Raising awareness in society through education about the problems patients with rare diseases face
- Organization of public events, congresses and meetings with the goal to educate and inform
- Support of research and education about rare diseases
- Organization of info centers for people with rare diseases
- Organization of cultural events for raising public awareness
- Distribution and printing of info-materials about rare diseases
- Social rehabilitation for people with rare diseases
- Finding funds for treatment of patients
- Documentation of patients with rare diseases

Important Issues

- Advocating for patients' rights.
- Raising awareness in society about rare diseases about physical and psychological consequences.
- Helping and informing families with rare diseases.
- Helping doctors to get information about diagnostics, treatment and rehabilitation of people with rare diseases.
- Communication with other organizations about rare diseases in R. North Macedonia and in the world.
- Communication with health-care institutions in R. North

Macedonia for better treatment of people with rare diseases. Meetings with relevant institutions: Ministry of Health, Health Insurance Fund, Ministry of Labor and Social Policy, Ministry of Finance, Macedonian Academy of Science and Art - Department of genetic engineering, Government of R. North Macedonia, Commission of Health in the Assembly of R. North Macedonia, Commission of rare disease at the Ministry of Health, Medicine Agency.

- Cooperating with the media to raise the level of awareness about our problems.

RARE DISEASES IN THE ORGANIZATION

The association of citizens for rare diseases LIFE WITH CHALLENGES, is founded by patients and parents that face life with Gaucher. Later, patients with many other different rare diseases joined the association. We accepted all of them as we believe that together we can help each other in advocating for better quality of life.

The association is representing patients and parents with rare diseases that are members in the association. At the moment there are over 90 diagnoses of rare diseases represented by the association: Gaucher, Phenylkeotnuria, Allagile Syndrome, Tyrosinemia, Lenox Gastaut Syndrome, Congenital Muscular Distrophy, Muscular Dystrophy Duchene, Muscular Dystrophy Becker, Congenital Cataract, Epydermolysis Bulosa, Carnitine plamitolitransferase deficiency, Adrenomyeloneuropathy, Williams Syndrome, RET Syndrome, Amyotropihi lateral sclerosis – Lou Gehrig's diseases, Myasthenia Gravis , Huntington disease, Porphyria, malignant paraganglioma Pheochromocytoma tumor, arthogryposis multiplex congenital, Fibrodysplasia ossificans progressiva (FOP), Multiple myeloma, Myelodysplastic syndrome, MPS 4 – Morqio Syndrome, Strümpell disease – Hereditary spastic paraplegia, Takayasu arteritis, Primary ciliary dyskinesia, Kartagener type, Mastocytosis, Juvenile dermatomyositis, Friedreich ataxia , Acute intermittent porphyria, Cystinuria, Stargart, Lowe Syndrom, Dextocardia Situs inversus, Neurofibramatozis, Phemgus Vulgaris, Wegener's Granulomatosis, Addison disease, Ehlers – Danhlos Syndrom, Alpha-1-antitrypsin deficiency, Aspergilosis, Erythrodermia Ichthyosiformis Congenital, Autosomal recessive dopa-responsive dystonia Tyrosine Hydroxylase deficiency, Alport Syndrom, Acute trasverse myelitis, Polimiozitis, Adrenogenital syndrome, Mastocitozis, Burgada Syndrom, Fanconi, Niemann Pick C, Chronic inflammatory demyelinating polyneuropathy, West Syndrome, Transverse Myelitis, Retinitis Pigmentosa, Carney Complex, Erythrodermia Ichthyosiformis congenita, Guillain Barreov Sindrom, Malignant Melanoma, Rendu-Osler-Weber disease - hereditary hemorrhagic telangiectasia(HHT), Systemic vasculitis-Sy.Churg-Strauss, Spinal Muscular Atrophy, Barter Syndrome, FAP - Familial Amyloid Polyneuropathy, Pulmonary Fibrosis, Amelogenesis Imperfecta, Achondroplasia, Kabuki syndrome, Fibrin-stabilizing factor deficiency, Klippel -Trenaunay syndrome … and others that will join us and ask for help and support.

The association does not represent rare diseases that are not registered members in the association.

Some groups of patients have their own associations and we encourage and support other groups to form associations since we think it is important for each disease to have association or at least a support group.

The association also is a member of the National Alliance for Rare Diseases of R. North Macedonia (17 organizations representing different rare diseases, patients' rights, and quality of life of families living with disability). LIFE WITH CHALLENGES is one of the founders of the alliance together with ESE and HEMOLOG, two other organizations which helped organize the founding conference for the alliance.

НАЦИОНАЛНА АЛИЈАНСА
ЗА РЕТКИ БОЛЕСТИ
НА Р. МАКЕДОНИЈА

A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as "rare" in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as "rare" in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as "rare" in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate

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WHAT HAVE WE ACCOMPLISHED?

Program for rare diseases at the Ministry of Health of North Macedonia

The first program for rare diseases at the Ministry of health dates back to 2009 and it included 3 patients with MPS 2 – Hunter syndrome.

There were no further changes until 2015. Some patients managed to get treatment through humanitarian aid, such as patients with Gaucher disease who were in critical condition and therefore, included in the Genzyme-Sanofi Aventis donation program thus receiving therapy for around 8 years.

From 2015 a register for RD was implemented at the Ministry of Health– about which more can be found in the program for RD from 2016.

In the new program for rare diseases from 2016, in the register for rare diseases are included the following rare diseases:

1. Diseases that can be treated with medicines.
2. Disease that are rare but there is no existent treatment

In the registry of patients with rare diseases solely the diseases listed in the ORPHAN list of rare disease are included – reviewed in July 2014

- http://www.orpha.net/orphacom/cahiers/docs/GB/List_of_rare_diseases_in_alphabetical_order.pdf

In the Registry of rare disease patients are only included diseases which affect one in 2000 persons, but which do not exceed 20 affected at the level of Republic of North Macedonia

Medicines are provided for the patients who are registered in the Registry of rare diseases through this programme. The provided medicines are necessary for the patients and represent an only choice of treatment of the rare disease, however, they are not on the positive list of medicines

For the realization of this programme, treatment of rare diseases with therapy and medicines which have not been previously applied in rare disease treatment, for the provision of the necessary equipment for diagnosis of rare diseases, providing education for the staff who diagnoses and treats the rare disease patients, it was expected that in 2016 total funds in the amount of 203.000.000,00 denars would be needed.

- Treatment of patients with a rare disease regardless of

insurance status 197.000.000.00

- Providing the necessary equipment for diagnosis and treatment of rare diseases 4.000.000.00
- Providing education of the staff who diagnoses/treats the rare disease patients 2.000.000.00

We were hoping that changes will be made in the program for 2017 since as patient organizations we all reacted to the limitation of the number of patients (20 per diagnosis), but until today there are no changes. And we reacted to the lack of further criteria and step by step explanation of how patients can be registered and get the needed treatment. We expect increased transparency regarding this. In 2019 the criteria was changed and it now says diseases where the number is not higher from 1 in 100 000 in the population. Other ways to procure medicines for rare diseases is through the Health Insurance Fund and through the budgets of the clinics that treat different rare diseases.

RARE DISEASE REGISTRY IN NORTH MACEDONIA

The registry for rare diseases consists of collected data for all diagnosed cases of people with rare diseases in a population.

The registration is a process of systematic data collection and analysis so that it can provide information on the number of rare diseases and the number of people affected by them.

Patients need to send a request through their doctors and the clinic, with the appropriate documentation about the diagnosis and treatment of the disease to the Ministry of health so that they can be included in the registry for rare diseases and get treatment if there is one. From 2018 the registry is in electronic format.

The commission for rare disease is consisted of doctors from the clinical center in Skopje. They gather to discuss about registering patients in the rare disease registry and they further discuss about the possible treatment and its provision to the patients who need it.

For now the Program for rare diseases has its limitations – it says that treatment will be provided to patients with rare diseases (where rare diseases are those with 1 in 2000 people as from the list of www.orpha.net , but only if they are not more than 20 in R. North Macedonia and receive no other treatment at all from others like from the Health Insurance Fund

or from the Clinics). We hope that this limitation will be withdrawn and all patients will get treatment. We also hope that drugs will be provided in a timely manner so that patients will not be left without therapy for long periods of time (3 months) because of administrative procedures.

In 2019 the Health Insurance Fund approved diagnostic- genetic analysis for inherited, rare and malignant diseases. With early diagnosis patients will have better prognosis and quality of life with early treatment and management of disease.

All these accomplishments are made with cooperation with other rare disease organizations in the National Alliance for rare diseases of R. North Macedonia.

MEDICINES FOR RARE DISEASES

Through the program for rare diseases are procured over 30 medicines for different rare diseases.

The budget of the Ministry of Health for the program for rare diseases from 2017 to 2019 is as following and it shows increase of finance:

- 2017 – 210 million MKD
- 2018 – 224 million MKD
- 2019 – 380 million MKD, but it is expected to reach 450 million MKD

In 2017- 2018 we started communication with the Minister Venko Filipce and we started negotiating to move forward each year.

In 2018 we got 3 new medicines, in 2019 we got 2 more new medicines.

In 2018 we also got a new law on parallel import that made it possible to get better quality of medicines and stop the import of falsified medicines and medicines with suspicious quality. After 2016 when there was a discovery of falsified drugs, this law helped in protecting all citizens not just people with rare diseases.

In 2018 we got 3 new medicines in the program and in 2019, 2 more medicines. We hope that this will continue forward.

HEALTH INSURANCE FUND

The Health Insurance Fund in 2014 informed the public about its decision

to start refund for a group of nutritive products for people with rare diseases like special flour, milk and other products. This decision is from 1st of August, 2014.

1. The Health Insurance Fund in 2014 informed the public about its decision to start refund for a group of nutritive products for people with rare diseases like special flour, milk and other products. This decision is from 1st of August, 2014.
2. Right to milk and oil for Alagille syndrome

- Group of metabolic diseases of amino acids (hyperleucin – isoleucinemia, hypervalinemia, izovaleric acidemia, methylmalonic acidemia, propionic acidemia)
- Group od metabolic diseases of fatty acids (adrenoleukodystrophy Addison schilder, deficit of muncul carnitine palmitoyltransferase)
- Group of metabolic disorders of amino acid transport (cystinosis, cystinuria, Fanconi-de Toni – Deber syndrome, Hartnup diseases, Lowe syndrome)
- Group of metabolic disorders of amino acids with Sulphur (Cistationuria, homocistinuria, metionimia, deficit of sulfite oxidase)
- Group of disorders of metabolic cycle of urea (argininemia, argininosuccinic aciduria, citrullinemia, hyperammonemia)
- Group of metabolic disorder of lizin and hydroxilizin (glutaric aciduria, hydroxilizinemia, hyperlysinemia, disorders of ornithine metabolism, ornithinemia type 1,20
- Group of disorders of glycin metabolism (hyperhidroxiprolinemia, hyperprolinemia, type1,2, nekton hyperglycinemia, sarcopenia)
- Group of other metabolic disorders of amino acids and y-glutamyl cycle

This news means savings for these families as they have a monthly expense of 4000 to 35000 MKD. This dates back to 2007 when for the first time the Health Insurance Fund started a refund for non-gluten flour for people allergic to gluten. In February, 2014 the Health Insurance Fund made this service available to patients with Rett Syndrome. Also non protein flour

and milk were added for people with Phenylketonuria. It is calculated that there are around 300 people with this kind of diseases and disorders that need special food, milk, oil and other products.

Epidermolisis bulossa patients finally got refund for special bandages in 2019, as promised in February, 2016.

HELP LINE FOR PATIENTS AND FAMILIES WITH RARE DISEASES 2015-2019

In the past years we managed to have a functional helpline for patients and families with rare diseases. Usually the communication is through telephone, e-mail, Facebook page, Facebook group, face to face meetings and patient and family gatherings where there is a larger number of patients.

Usually we have more than 2 calls on a daily basis, not just on work days but also on weekends and holidays. Sometimes we get calls from people who face other kinds of diseases and we try to direct them towards reliable organizations.

With a goal to help more we are in communication with organizations from the region like Croatia, Serbia, Montenegro, Bulgaria, and Bosnia and Herzegovina. We are all enthusiastic and persistent in our work to support patients. We also organize regional conferences, meetings, we share best practices, contacts from medical professionals, and we advise and support each other to achieve more for the families who face rare diseases.

The request for help are usually those bellow:

- Do I have a rare disease or just an ordinary one?
- Where can I find information? / Can you translate information for my diseases?
- Is there a medicine?
- Is there any other treatment available or not?
- How should I be registered to get a drug?

- Do I have the right to social aid?
- Do you know someone with this diseases?
- Can you connect me with others like me?
- Do you know doctors that can provide an explanation about my disease?
- Which institution should I contact?
- How to write a letter to an institution?
- Can you take my documents to the institutions in my name?
- Can you help me with gathering documentation?
- Is stem cells treatment useful?
- Who in the Health insurance is responsible for rare diseases?
- Who in Ministry of health should I contact?
- Is my special food for my disease refunded? / How can I get refund?
- Is the clinic responsible for provision of my drugs?
- Who decides if I get a drug or not?
- Do I qualify for social aid?
- How long will I need to wait to get a medicine?
- Is there a clinical study on my diseases?
- Can you please read and explain how a clinical study works?

We try to answer immediately if we can or in 2-3 days. If we do not get an answer until then, we call and say that we need more time. If we are still not able to help we try to connect and communicate with other organizations and institutions.

Also through the helpline we managed to help with the foundation of 5 new associations. We gather patients with the same disease, they have a meeting and decide on further actions. Of course we helped them in documentation and registration, we help with project writing, in letters to institutions, we give contacts, we arrange meetings and other similar support. For now we helped, Wilson, Thalassemia, Pulmonary hypertension,

Muscular Dystrophy, Spinal Muscular Atrophy Association and for people with FAP. Additionally we also helped in the formation of the association for multipl sclerosis. Sometimes they do not form an association but an informal group within the association Life with challenges.

We also invite new organizations to join the National Alliance for Rare Diseases of R. North Macedonia. We think that cooperation is very important if we want to achieve our mission and goals.

Another activity is grouping problems that come from a specific area. For example when we see that there are many problems in orthopedic devices, we gather info from patients and organizations and then we arrange a meeting with the Health Insurance Fund with a goal to overcome the challenges and find a mutual solution.

Our greatest accomplishment is definitely connecting patients and families. We have had many successful stories and many happy families because of this activity. When you get a diagnosis it is not easy to accept it and continue further with your life. But when you talk to someone who has already had this diagnosis for years it is easier to imagine how your life will be like in future. Also you can exchange experience and knowledge and overcome challenges together.

At the end of 2018 we received a donation from Foundation Trajche Muaketov, ALKALOID AD Skopje to continue our work regarding the Helpline and we managed to sign a memorandum of cooperation with psychologist Ivana Hadzivanova, who is head of NEOKOTREKS so that patients and families have psychological support that they need and in 2019 we also have a social worker supporting the patients. To improve access to information on rare diseases, which is very important, we signed a memorandum of cooperation with the association LIFE from Serbia. They started a web page with information on rare diseases in Serbian language and they included a forum for discussion for patients and for medical professionals. Now we will add translation in Macedonian language and the forum will be open to our patients and medical professionals as well. This way we are certain that the help and support we are offering is on a much higher level. And again the most important thing is cooperation.

RARE DISEASE AWARENESS

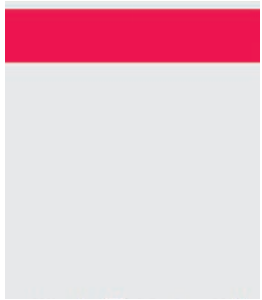
RARE DISEASE DAY

We started marking rare disease day since 2012. From 2015 we are marking the day together with the organizations from the National Alliance for Rare Diseases of R. North Macedonia.

Every year we manage to organize more events for raising public awareness with the help of our members and with cooperation with other organizations.

In 2014 we got the support from the wife of the then USA ambassador in North Macedonia Ms. Mary Jo Wholers and in 2015 and 2016 we had the support of the First Lady of North Macedonia – Ms. Maja Ivanova. Further we had support from the director of the Children Clinic – Ms. Aspazija Sofijanova, then the director of the Health insurance fund – Ms Maja Parnardzieva Zmejkova, also from the Macedonian Academy for Science and Art we had Momir H. Polenakovikj and Dijana Plasheska Karanfilska. Every year we got more and more support from institutions also including Ministry of health and Ministry for social policy. Also we got support from medical professionals and researchers that work in the field of rare diseases.

The part of "LIFE WITH CHALLENGES" in Rare Disease Day, 2016 which we are very proud of was a promotion of the study for rare diseases that was realized in 2015 and can be downloaded on our web page in the tab - Brochures.



Poster from the study was presented at European Conference for Rare Diseases 2016.

We are proud to say that rare diseases get more attention in the media and at the institutions in R. North Macedonia. We hope that more events will contribute towards acceptance from the public and acknowledgment from the institutions.

Rare Disease day, 2015, City Mall, Skopje

In 2016 we managed to organize events in Bitola with the help of Hepar Centre Bitola, in Prilep with the support of Jasminka Hristoska and in Ohrid with the support of Gordana Loleska and the Red Cross from Ohrid.

Rare disease day, 2015, Youth Cultural Center, Skopje

Rare disease day, 2017, EU Info Center, Skopje

Round table on rare cancers, 28th of October, 2016, Skopje, North Macedonia with support from Serbia and Bosnia and Herzegovina and the association for cancer BORKA from North Macedonia

Rare disease day, 2018, Skopje, EU info center

SPECIAL THANKS TO OUR MOST ACTIVE MEMBER – GORDANA LOLESKA FROM OHRID

We would like to congratulate Gordana Loleska for doing as much as possible and more to raise awareness on rare diseases in North Macedonia and internationally. After her statement, you can read about some of the events she implemented - they are as amazing as she is. Every organization needs a person like her.

> „My story starts 4 years ago when the condition of my child got a diagnosis, a rare disease – Alport syndrome. And it was a shock to me – a rare disease that has no available treatment in the world. I was depressed, desperate and very sad. I followed the advice of Prof. D-r Tasik, and Prof. D-r Kuzmanovska and started to be more active in support groups for rare diseases. In one of those groups I met Vesna Aleksovska, a woman who was my first support and I learned a lot from her about raising awareness, about organizing events to support families with rare diseases, about many different rare diseases … this woman today is my friend and the wind in my back … My fight, our fight … is never over … we continue further with a goal to help at least a little bit and change the world for better … people with rare diseases deserve that. "
>
> *Statement, Gordana Loleska, member and activist in*
>
> *LIFE WITH CHALLENGES*

POSTAL STAMP FOR RARE DISEASES

In 2015, Gordana Loleska, employed at the post office in Ohrid, started an initiative for creating a postal stamp with the logo of rare disease day to help in raising awareness for rare diseases in North Macedonia. This initiative was supported by many medical professionals in North Macedonia who sent a letter of support to the Direction for philately to the Director Mr. Elez Elezi.

We did an internet search and we realized that this kind of stamp will be first of its kind – dedicated to Rare Disease Day to all the Children with Rare Diseases and it is a great initiative for the Macedonian post office, an accomplishment on world level.

In 2016 the post accepted this initiative and it was promoted in October, 2017. The name of the stamp is Children with rare disease. From 2019 forward, every year there will be a new stamp with an individual rare disease, to raise awareness. The first will be for Alport syndrome.

We are very proud of Gordana Loleska who enthusiastically took this idea forward. We are also thankful to the general director of the post office Mr. Fadis Redzepi, to the director of the sector for philately Mr. Elez Elezi, to the director of the post office in Ohrid Mr. Sejfula Hani and to the council

for philately at the post office of R. North Macedonia, with its president Ms. Vasilevska Sonja.

In June, 2019, the post published a new postal stamp dedicated to the rare disease Alport Syndrome.

Rare Disease Day Flag under water for New Year, December, 2016 and 2017, Ohrid, North Macedonia

Climbing the highest mountain in Western Europe – Mont Blanc in France in support for rare diseases in North Macedonia

Rare diseases at Ohrid running, June, 2017

On 8th September, 2017 the Independence Day of R. North Macedonia the International Athletic Marathon started in Ohrid.

The Minister of finance Mr Dragan Tevdovski, participated in the Marathon in support of rare diseases.

Santa Clause race, Ohrid, December, 2017

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SPECIAL THANKS TO
MEMBERS/ ACTIVISTS OF
LIFE WITH CHALLENGES

Dragan Dimitrijoski, member and activist

Dragan is one of the first members of the association. As a father of a child with Phenylketonuria – PKU, he was one of the first families that spoke in the media about the challenges of living with a rare disease.

Together we managed to get the special food for PKU refunded (not just for PKU but also for other rare diseases). And in 2015 we managed to get Kuvan on the list of provided treatment for rare diseases.

The PKU group of families is in communication and they support each other so that they have a better quality of life. Dragan moved away to Sweden with his family, but we are not going to forget him. He achieved a lot and he showed that we need to be brave and persistent.

> *Statement Dragan: I am happy that we are not forgotten even though we moved away from North Macedonia. It makes me hopeful to see that the association is working and overcoming different challenges through the years. I would like to say thank you, because before LIFE WITH CHALLENGES rare diseases were a taboo, all doors were closed and getting a medicine in the country was science fiction. Today that is very different.*

Blaze Arsov, father of a child with Alagille syndrome and a member/activist

Blaze was one of the first parents to join in our association together with Dragan. He was active in events, discussions and meetings. And because of his profession as a physiotherapist he also took interest into learning more about physiotherapy and rare diseases. We are proud that he managed to organize different events (educative, informative and awareness raising) on physiotherapy in patients with Cystic Fibrosis. We hope to further educate more physiotherapists on different rare diseases – specific on neuromuscular diseases where exercise is one of the most important parts of treatment. Blaze continues to be active in our association and in the National alliance for rare diseases. Also he is the president of the Macedonian society for physiotherapy.

Josif Mishevski, patient with ichthyosis and a member/activist

We want to say thank you to Josif as when he joined our association he became very active, attending events, helping out in organization of activities, meeting with institutions, starting cooperation with the Ministry of Labor and Social Policy. Then he continued to organize events and gather patients in Gostivar for rare disease day, and he continued with activities supporting activities of special needs organizations.

Today he works in the Ministry of Health in the pharmaceutical sector. We are happy to have him as our member and we hope will have even more success in future.

> *Statement Josif: My activism in LIFE WITH CHALLENGES started because of my rare disease. A disease that I face every day and I consider it my friend. After I finished studying, I had enough reasons to dedicate my time to something new, and find reasons to stay positive and strong. My life was filled with darkness and at the end of the tunnel I saw a bright light – this was LIFE WITH CHALLENGES. After I saw what the vision and mission of this association is, I felt I had to contribute toward early diagnosis, treatment and disease management in our country. But also I knew we had to do more on overcoming the stigma and discrimination. After I joined LIFE WITH CHALLENGES, my life became more active, more positive, and my views wider. I became a better person with a strong spirit!*

Kire Sazdovski, patient with pemphigus vulgaris / member/ activist

Kire joined us in the beginning of our work. He was not always able to attend events and be involved in organization of activities but he was always here to write an inspirational and motivational letter.

No one will forget his motto – I accepted the disease, but not the defeat! We are all fighters, we are all together. Thank you Kire for your inspiration and support through the years.

> *Statement Kire: I would like to express my respect towards the continuous fight for better life of patients with rare diseases. I wish that there was no need to form organizations and all people to be healthy and have the appropriate care and treatment. But life is full of challenges and this is why LIFE WITH CHALLENGES was formed. I can only say thank you to Vesna, as she tackled a lot of problems since the beginning, fighting for better quality of life of families facing life with a rare disease. We should always give support to people who fight for a better tomorrow.*

Veneta Jankova, patient/member/activist

Veneta is the person who has a lot of information on Myastenia Gravis. As a patient and as a medical professional she had opportunity to get a lot of information on this disease, and she is always here for the parents and the patients to share the information and exchange best practice. We are thankful that she is still positive and energetic, besides the health problems she has faced. And we are happy that she is here to support families.

Statement: Help from professional and moral and psychological support is very important for patients and parents who face something new and unknown. It is very important to learn how to handle all the problems and challenges that come with a rare disease. It is of paramount importance for patients to know that they are supported and that possibilities are here for them. They need to be encouraged, to share between themselves and to support each other. Every challenge should be accepted as it is and go a step forward into the future. Head up and let's do more, while we hold hands to feel more encouraged and stronger.

Denica Velkovska, parent/collaborator/activist

Denica appeared in the rare disease day event in 2013. She stood up from the audience and she said that she does not trust patient organizations as they have done nothing for her child. After that we sat together and we talked for a long time as we agreed that we need to fight together for patients with Spinal muscular atrophy. There was no medicine at the time for Jane, her child. But when the medicine got approval in Europe together we fought to get treatment for patients in North Macedonia. Thanks to her enthusiasm and persistence now people with SMA in North Macedonia will have treatment. Together we are stronger, together we can do more. Thank you Denica for being a wonderful mother, and a strong woman.

Daniela Mirhcevska, parent/member/activist

Daniela joined the association as her daughter was diagnosed with Juvenile dermatomyositis. Both of them are amazing people. They actively got involved in raising awareness and fight for a better quality of life. These are people that make the world a more beautiful place to live in. Daniela was also involved in the resource center for children with special needs and other associations that advocate for children with rare diseases and special needs.

Biljana Pavleska, patient/member/activist

Biljana has not one but more rare diseases. It started with Churg Strauss, and then fibrosis, and autoimmune disease. She never gives up. I can count on Biljana that she will call at least once a month to give update on what is going on and to share good news. Even though she is facing a difficult condition she always finds strength to smile and motivate others to work more and do more. We are thankful that she is a part of the rare disease family in North Macedonia.

Nebojsha and Katerina Petrevski, parents/members/activists

This wonderful family joined the association as their child got a diagnosis Epidermolysis Bulosa. The rare disease made them special. They support all events on rare diseases, they actively speak in public about the challenges, they help out with organizing events and taking photos as they own Procam studio. Together with Josif from Gostivar they organized events for rare disease day in Gostivar that were very successful. We are thankful to have them in our rare family.

Viktorija Penova, patient/member/activist

Viktorija is one of our first members in the association. Facing a very rare disease – FOP, from which her muscles become bones, she still found strength to live her life with as much joy as possible. She always found a way to come to events and make inspirational speeches. Viktorija motivates a lot of patients and we are happy to have her.

> *Statement Viktorija: To face this rare disease was one of the most difficult things in my life, and it is the most difficult thing that can happen to a family. In all that process it was very important to have my family and friends around me. I needed a long time to accept the diagnosis as a part of me. It was helpful to learn that I am not alone, that others are like me. Talking to people with the same diagnosis helped me understand that I am not alone, and I gathered courage and hope that life is still possible. I was lucky to meet 4 people that share my disease, the feeling is amazing. With persistence and desire I managed to finish high school and now I am in college. I am proud of my success and happy to have my family around me to support me in everything that I do in life.*

Ivana, patient/member/activist

Ivana joined the association because she was facing life with epidermolysis bulossa. That was not an obstacle for her, as she always found a way to come to events, to communicate regionally and internationally and to fight to find a way to get better quality of life for patients with EB. Thanks to her persistence, today patients with EB have refund for the needed plasters for care of the skin.

Statement: A diseases is nothing when you have treatment. Not having treatment is difficult, it is even more difficult when there is treatment but you cannot buy it and provide it. All the stress from not being able to get the therapy we need is even worse than the disease itself. Rare diseases are not diseases but conditions, something you can live with. I have skin problems because of

the disease, but with special creams and plasters I can have more normal life. And I have to say that it is very important for us patients to meet with each other and together to fight for our rights.

Marija Dimovska, volunteer/ collaborator

> I have been involved with Life with Challenges since its conception. It is very elating to see this organization grow and help so many people. They have become a major stakeholder in raising awareness not only for rare diseases but also for other health care issues. With their humane and holistic approach they are the bright star that leads the way for many patients and families living with a rare disease.

Anja Bosilkova-Antovska, patient/collaborator/activist/Wilson Macedonia

> *Many years after diagnosis I thought that I was alone and that I have no one to share my problems and challenges with. And then LIFE WITH CHALLENGES organized the first meeting for Wilson disease patients in North Macedonia. This was the moment when I got a new family so that together we can overcome the challenges in front of us. With the support of LIFE WITH CHALLENGES in 2014 we founded the association for Wilson diseases and in few years we accomplished a lot. We had many activities on education, information and raising awareness. Through all these years LIFE WITH CHALLENGES had a great role in our work, always supporting us and advising us when needed. Together we continue to work for better quality of life of patients with rare diseases, for systemic solutions to our problems. We hope to continue the successful cooperation in the future.*

Special thanks to **Milenka Sarovic**,

We would like to say special thank you to Milenka Sarovic, as she personally got involved in supporting patients with rare diseases even before we had an association in 2009. She supported us with great enthusiasm and optimism and she continued to help us until today. In the last 5 years, she took the rare disease flag on each mountain top that she climbed as she wanted to support raising awareness on rare diseases. People like Milenka are rare people and we are honored to have her on our side.

We are thankful to the European organization for rare diseases – EURORDIS and International Gaucher Alliance – IGA, as they supported us in our work and thanks to the many conferences and meetings we managed to achieve good communication and cooperation with different

countries and to provide exchange of experiences and best practices.

We are thankful to our many collaborators from other associations like: Biba Dodeva, Violeta Tomovska, Natasha Maslesha, Milan Mishkovikj, Pavlinka Baliska, Borjan Pavlovski, Biljana Vlastimirova, Rebeka Jankovska Risteski, Iskra Doneva, Gjurgjica Kjaeva, Cveta I Marija Nakjeski, Dragi Lulevski, Natasha Anjgeleska, Natasha and Verce Jovanovski, Maja Dimikj, Anja Bosilkova Anovska, Lile Rajceska, Aleksandar Petrovski, Pepo Levi, Martin Pandovski, ... and many others that helped in our efforts to fight for rights of patients with rare diseases.

A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as “rare” in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as “rare” in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic disease. No single cut-off number has been agreed upon for which a disease is considered rare. A disease may be considered rare in one part of the world, or in a particular group of people, but still be common in another. The US organisation Global Genes has estimated that more than 300 million people worldwide are living with one of the approximately 7,000 diseases they define as “rare” in the United States. | A rare disease is any disease that affects a small percentage of the population. In some parts of the world, an orphan disease is a rare disease whose rarity means there is a lack of a market large enough to gain support and resources for discovering treatments for it, except by the government granting economically advantageous conditions to creating and selling such treatments. Orphan drugs are ones so created or sold. Most rare diseases are genetic and thus are present throughout the person's entire life, even if symptoms do not immediately appear. Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate isomerase deficiency is considered the rarest known genetic Many rare diseases appear early in life, and about 30% of children with rare diseases will die before reaching their fifth birthday. With only three diagnosed patients in 27 years, ribose-5-phosphate worldwide are living with one of the approximately 7,000 diseases they define as “rare” in the United States. | A

TRADITIONAL SPORTING ACTIVITIES PROMOTING HEALTHY LIFE AND RAISING AWARENESS ON RARE DISEASES.

The Asscent of Korab, September, from 2013 to 2018

Skopje Marathon – Running for rare diseases (from 2013 to 2017)

COOPERATION WITH DOCTORS AND RESEARCHERS

From the beginning we started to communicate with doctors and we have developed a special bond between us as a patient association and the University children clinic in Skopje as most of our members were children, patients treated and monitored in this clinic.

For 6 years in a row our organization is a participant and co-organizer of the MEETING OF South East Europe FOR RARE DISEASES, NOVEMBER, 2012 - 2017

The Macedonian Academy for Science and Arts had the opportunity to organize 6 meetings in a row, every year in November, from 2013 we were included in the program and the organization of the conferences. We earned our place on the table with the stakeholders. Nothing for us, without us – is a great patient motto that we continue to promote.

In 2019 for the first time we are organizing together a meeting for rare diseases for doctors, researchers, and also for patient representatives. We proved that together we are stronger.

COOPERATION WITH MACEDONIAN MEDICAL STUDENT ASSOCIATION – MMSA

In 2019 together with MMSA, we organized lectures for rare diseases. We are thankful to the organizers led by Monika Stojanoska, as they successfully organized this event where over 300 students were present. The interest of the medical students shows hope for better future for the families that face life with rare diseases.

COOPERATION WITH OTHER ORGANIZATIONS AND MEMBERSHIP IN ALLIANCES

Internationally we are actively participating in the board of directors of the International Gaucher Alliance – IGA, in the Drug Information, Transparency and Access Task Force in EURORDIS, and we are members of several organizations internationally and nationally: International Gaucher Alliance, EURORDIS, International Pompe Alliance, MDS Alliance, International Alliance of Patients Organizations, National Alliance for Rare Diseases of R. North Macedonia, Alliance for patient organizations – APO in North Macedonia, an organization that is working on helping patients and families that face cancer. We also cooperate with organizations from the Balkan countries from Serbia, Croatia, Bulgaria, Bosnia and Herzegovina, Albania, Montenegro, Kosovo and Slovenia.

Why do we think that cooperation is important?

- Sharing knowledge in patient advocacy.
- Sharing tools and best practices with patient organizations.
- Learning how other organizations in the world accomplish their goals.
- Getting support in policy development.
- Getting support and new ideas for activities about raising public and institutional awareness about the problems and challenges of the patients and families.
- Access to workshops, meetings, seminars and training on subjects important for development of strong patient advocates.
- Access to new information of research and development of new medicines, new policies and new ways to help and support patients

Conferences:

Second regional meeting of Gaucher patients and first regional meeting for lysosomal diseases, Sarajevo, Bosnia and Herzegovina, 5-7 April, 2013

Second Balkan Conference for rare diseases – "Better communication – better treatment", 20 – 22 April, 2013, Sofia, Bulgaria

EURORDIS Membership Meeting, May, 2013, Dubrovnik, Croatia

Patient Meeting Bulgaria – North Macedonia, Skopje, 7 September, 2013

NORBS – National Plan Conference, Belgrade, Serbia, 5-7 December, 2013

DITA Task Force Meeting (Drug Information, Transparency and Access), London, UK, 8-10 December, 2013

EUPATI – European patients' academy on therapeutic innovation – 2014 – 2015

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SPECIAL THANKS TO OUR
SUPPORTERS THROUGH THE YEARS

Special thanks to associations that we cooperate and work with through the years

1. Skopje Night Running
2. Association for cancer BORKA
3. Association HEPAR center Bitola
4. Association LIFE SPARK
5. Association BUTTERFLY
6. Red cross of Gazi Baba, Skopje
7. Red cross of Ohrid
8. Youth Educational Forum
9. Na Tocak
10. To4ak
11. Alliance of patient organizations of North Macedonia
12. Association for hemophilia HEMOLOG
13. Association for Hereditary angioedema
14. Association for emancipation, solidarity and equality for women
15. Association for rare neurological diseases KOKICNINJA
16. Association for pulmonary hypertension MOMENT PLUS
17. Association for happier life of people with mental and physical dysfunction NEW SPARK
18. Association for rheumatism and arthritis NORA
19. Association for Down syndrome – Trisomy 21
20. Association for people with dysfunctions, invalidity, rare diseases and special needs – GIVE US WINGS
21. Association for Wilson disease
22. Association for hemophilia Macedonia
23. Association for hemophilia HEMOWIL
24. Association for thalassemia
25. Association for Spinal Muscular Atrophy – STOP SMA

Gratitude to our donors and sponsors

It was not easy for a small association as ours to organize many events and to maintain cooperation and communication with all stakeholders in the rare disease field.

We are thankful for the support that we got from many private companies, public institutions, pharmaceutical companies, and individuals who supported our activities in the past 10 years and we hope to continue cooperation further as we have much more to do in the next 5 to 10 years. We know that together we will achieve a lot.

We have to say at first special thanks to Genzyme-Sanofi Aventis as they were the first pharmaceutical company that supported our activities with a grant in 2012. And this is the company that donated treatment for 5 patients with Gaucher disease from 2009 to 2015. Also they helped with diagnosis, as all patients were diagnosed through them.

I must not forget to say how grateful we are to Concept Marketing and Communications as they transformed our identity with a new logo, a web page, and a a way of organizing events,they have been there with us from the beginning, holding our hand to manage successful realization.

After that I have to mention Celgene International and say thank you for being with us from 2014. Also they were donating treatment for patients with Myelodysplastic syndrome and with that enabled a future for them.

There are many more supporters each of them special and unique in its own way as they enabled us as an association to grow with integrity and credibility and they were with us, supporting us in requesting changes in the health system and the social system so that patients with rare diseases can be included, so that patients have access to diagnosis, treatment, control and management of the disease, and social services.

We are thankful to Pfizer for supporting our activities and also for donating financial resources to the Macedonian Academy for Science and Arts for genetic diagnosis for Gaucher Disease and FAP - Familial Amyloid Polyneuropathy.

> *Statement Pfizer: "We would like to congratulate LIFE WITH CHALLENGES patients' organization on the 10th Anniversary of the great collaboration and broad engagement on different country levels for their support in awareness and increased medicines access to the patients. For advanced treatment possibilities the visibility, pervasiveness and dedication to the patients best therapeutic options and well-being is extremely important. We are counting on the team*

that it will continue with all the professionalism and assertiveness also in the future. We are HERE together to listen, to learn and to make difference. We are HERE for patients."

Biomarin supported our activities and helped patients by donating financial resources for infusion pumps so that the medicine can be administered more easily.

Further we would like to thank Hoffman La Roche, Medis and Novartis for financial support of our activities for raising awareness, conferences for cooperation and raising institutional awareness and support of families with rare diseases.

Statement Novartis: "At Novartis, we explore new ways to improve and extend people's lives. We understand that our therapies are part of the solution and we need to work closely with patients to understand their journey in the healthcare system. We look for insights to provide solutions for patients and their relatives. Novartis proves to be a trusted partner of healthcare professionals, patients, institutions and society..."

Media planet as a company donated 20% of the sales of their children magazines to our association from 2016 which helped us to realize a lot of activities for support and help through the helpline for rare diseases.

Our help line was improved and continued to grow into info center for rare diseases with the donation from Foundation Trajche Mukaetov, ALKALOID AD Skopje from 2018.

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