Живот со Предизвици / Jeta me Sfida / Life With Challenges

Activity report for 2015

In 2015 Life With Challenges realized many activities for raising public awareness, strengthening patients, cooperation and networking with other organizations on national and international level and communication with the relevant health institutions in Macedonia.

Internationally we continued to actively participate in the board of directors of the European Gaucher Alliance - EGA, in the Drug Information, Transparency and Access Task Force in EURORDIS, and we became member of the International Alliance of Patient Organizations – IAPO - https://www.iapo.org.uk/ and the MDS Alliance - http://www.mds-alliance.org/.

Our most important accomplishment is the **launch of the new law for financing the Program for Rare Diseases at the Ministry of Health**, which was adopted in January, 2015. This is a hope for all untreated patients with rare diseases. The program until this year had 1, 5 million EUR, with this law it has additional 4, 5 million EUR – in total 6 million EUR. On December 16th the Prime Minister of Macedonia Mr Nikola Gruevski gave the following statement:

"The challenge of treating patients with rare diseases is a world challenge. Although they influence 1 in 2000 people they are a challenge for many families and they are before all a life challenge for the patients … our task is to try and find a way to overcome those challenges and give support to the families, we need to fight together for the health of our citizens. We have a task to find a way to finance the fight with rare diseases in an appropriate way. That is why in Macedonia we found a systematic solution that is in accordance with EU countries, we changed the Law for taxes on cigarettes, and from now on 1 MKD from one packet of cigarettes will go directly to the program for rare diseases in Macedonia. We estimate that 250 million MKD will be gathered to treat the patients. We need to continue working together, the fight of one citizen is a fight for all citizens."

In February, 2015, the Ministry of Health started with the development of a **Registry for rare diseases**. The lists of patients send by organizations for rare diseases and by clinics/hospitals were reviewed by a Commission of specialists' doctors and then approved by the Commission for rare diseases. All patients on the lists were called for the review to bring documents for diagnosis and needed treatment.

With this step it was realized that we have another problem in Macedonia and that is in the field of diagnosis. Not all patients had confirmed diagnosis since for some diseases there is no available diagnostic material or technical equipment in the country. Unfortunately there is also no reimbursement for patients that get diagnosis abroad. This is a problem that we are going to emphasize in the next year.

The registry of patients is led by a special department in the Ministry of Health and we continue to send more patients to be registered. In the next year we will focus our activities on cooperation with clinics and hospitals to raise awareness among doctors about the existence of the registry so that they will register other rare diseases and other patients.

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In April, 2015 the **tender for medicines for rare diseases** was announced including 12 rare diseases and 21 new medicines. And then there was a second tender in October including medicines for cystic fibrosis that were previously provide by the Health Insurance Fund through the Children Clinic in Skopje.

Number	Generic name of the medicine / Rare disease	Strength of the medicine	Form of the medicine	Quantity
1	Nitisinone – for Tyrosinemia	5mg	Capsule	1080
2	Imiglucerase – for Gaucher Disease	400 IE	Concentrate for infusion	1784
3	Coagulation factor XIII concentrate (human) - for deficit of coagulation factor 13	250 IU	Concentrate for infusion	24
4	Coagulation factor XIII concentrate (human) - for deficit of coagulation factor 13	500 IU	Concentrate for infusion	18
5	C1 esterase inhibitor human – for Hereditary Angioedema - HAE	500 IE	For injection	70
6	Sapropterin – for Phenilketoyria - PKU	100mg	Tablets	3000
7	Teriparatid – Neuromuscular disease	250 mcg/ml	For injection	15
8	Sirolimus – for Tuberous sclerosis	1mg	Tablets	3600
9	Everolimus – for Tuberous sclerosis	2.5 mg	Tablets	1440
10	Stiripentol – for Tuberous sclerosis	250mg	Tablets / Capsules	2880
11	D -penicilamin – for Wilson disease	250mg	Capsules	14180
12	C1 esterase inhibitor recombinant – for Hereditary Angioedema - HAE	2100 IE	For injection	48
13	Sildenafil – for Pulmonary Hypertension	50 mg	Tablets	3780
14	Sildenafil - for Pulmonary Hypertension	20 mg	Tablets	1080

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15	Zink acetate - for Wilson disease	50 mg	Tablets / Capsules	1500
16	Zink sulfate - for Wilson disease	50mg	Tablets	4320
17	Tetrabenazine - for Hauntington disease	25mg	Tablets	3600
18	Vigabatrin - for Tuberous sclerosis	500mg	Tablets	11000
20	Rilusole – for ALS – Lou Gehrig's disease	50mg	Tablets	2160
21	Tocilizumab – for Juvenile Arthritis	200mg/10ml	For injection	72
22	Coagulation factor XIII (human) - for deficit of coagulation factor 13	250 IU	For injection	36
23	stiripentol– for Tuberous sclerosis	250mg	Tablets / Capsules	2.880
24	Zink sulfate– for Wilson disease	50mg	Tablets	4.320
25	elosulfase alfa – for MPS 4 – Morquio syndrome	5mg/5ml	For injection	480
26	colistin (colistimethate)– for Cystic fibrosis	1 MIE	For injection	27.600
27	dornase alfa (desoxyribonuclease) - for Cystic fibrosis	1 mg/ 1 ml 2,5 ml	For inhalation	23.400
28	tobramycin – for Cystic fibrosis	300 mg/4ml 300 mg/5ml	For inhalation	10.080
29	Vancomycin – for Cystic fibrosis	500 mg	For injection	800
30	vancomycin – for Cystic fibrosis	1000 mg	For injection	1.600
31	meropenem– for Cystic fibrosis	500 mg	For injection	1.200
32	meropenem– for Cystic fibrosis	1000 mg	For injection	2.400
33	amikacin– for Cystic fibrosis	500 mg/2ml	For injection	4.800

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This list of medicines is not final. The Ministry of Health promised that they will review this list as new patients are registered from the existing rare diseases or other rare diseases and they will add new medicines or new quantities of medicines to satisfy the needs of the patients. As an organization we will continue our cooperation with the Ministry of Health and we will advocate for this promise to become reality.

1. Opening an office, 2015

This year with the support of our sponsors we managed to rent an office space. The office space is shared with the Non-government organization for rheumatism and arthritis – NORA. We decided that this way we will strengthen our cooperation in the field off rare diseases because there are many rare diseases in the field of rheumatology that need support and advocacy. Also this way we are able to work on projects together and to share the administrative burden in maintaining an office. We hope to continue this cooperation through the next year.

2. Rare Disease Day, 28th of February, 2015

For rare disease day Life With Challenges realized an event following the moto “Day by day, hand in hand”, under the organization of EURORDIS where the organization is a full member since 2013.

This year we organized the event with the support of 11 rare disease organizations, members of the National Alliance for Rare Diseases founded in May, 2014. Together we realized a press conference, where we had a speech from the First Lady of R. Macedonia – Ms Maja Ivanova, who continued to support our activities in the field of rare diseases.

After the press we opened the exhibition - Embrace Rarity and we had a cocktail for the invited guests. The special magazine for rare diseases was also promoted at the event and shared with the public. Promotional materials (flyers, posters, brochures) from different organizations were also shared at the event. For the first time in Macedonia, RDD was also marked with an event in Gevgelija supported by the Organization for Pulmonary Hypertension Moment Plus.

The magazine “Face to Face” is available for download on our webpage - Face to Face - English version - download.

Poster and flyer in Macedonian and Albanian for RDD:

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Mrs. Maja Ivanova greeted the patients and their families whose everyday struggle helps to raise the awareness on rare diseases. She urged not to emphasise the differences, but to build a better life together: *"Rarity in the world represents uniqueness and prestige. Rare diseases are also unique, and patients with a rare disease are equally unique and their lives precious. Because every life is equally valuable it is important to have early diagnosis and suitable treatment for rare diseases. I was priviledged to be honorary patron of the event orgaised by EURORDIS in Brussels where together we discussed the support in the area of research, treatment, early diagnosis as well as support for the patients. Rare diseases should be a priority both on European and national level."*

At the event ***Certificates of Gratitude*** were awarded to the following individuals, state institutions and private companies: Mrs. Maja Ivanova, First Lady of R. Macedonia, Mr. Nikola Todorov, Minister of Health of R.Macedonia, Mr. Momir H. Polenakovik, Macedonian Academy of Sciences and Arts, Prof. MD Aspazija Sofijanova, Agency for Medicines, R.Macedonia, Foundation Open Society Macedonia, US Embassy, Macedonia, Genzyme –Sanofi Aventis, Central European Initiative- CEI, Hoffmann-La Roche, Vinary Brzanov, Brewery Star Grad, Foundation Makedonika, ONE, VIP, Kontura, CONCEPT Marketing and Communications, ALKALOID KONS Ltd, ALKALOID AD Skopje, Prof. MD Aco Kostovski, Prof.MD Zoran Guchev, Prof.MD Vesna Grivchev Panovska, Ass.MD Ivan Milev, Prof. MD Velibor Tasikj, Ass. MD Biljana Chonevska Jovanova, Nurses from the haematology ward, University Clinic for Children's Diseases, NaTochak, To4ak, Red Cross of Municipality Gazi Baba, ZPK Korab, Natasha Dimitrievska Krivoshev, Zoran Mihajlovski, Dragan B.Kostikj, Valentina Bachvarovska.

The exhibition - Embrace Rarity, was also presented on other events after Rare Disease Day such as the Hereditary Angioedema Congress at the Medical University and the Congress for Physiotherapy in the Macedonian Academy for science and Art.

This exhibition – Embrace Rarity - was prepared for Rare Disease Day, 2015, from the platform for activism "Lice v Lice", in cooperation with "Holistic", the Association of citizens for rare diseases Life With Challenges and the National Alliance for Rare Diseases of R. Macedonia. Below are the pictures from the exhibition together with the text that is explaining them.

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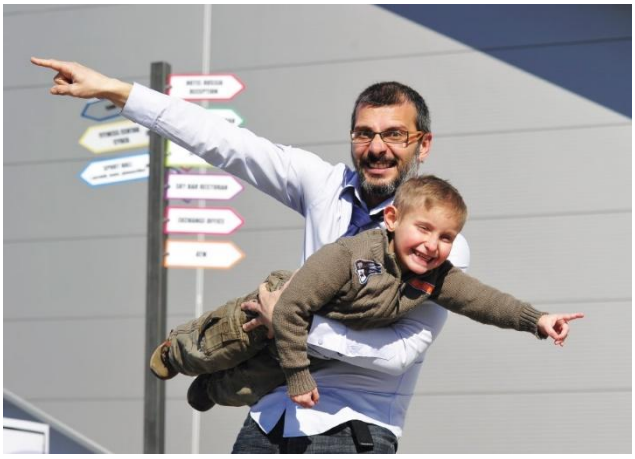
Ognen Janeski and Azemina Kurtishi - "Rocketing"

Azemina is the first patient with a diagnosed rare disease Gaucher who has received appropriate therapy from the state.

Without it, this disease in its last stage brings the patients in a state where they cannot move, with discomfort and growing of the liver, osteoporosis and foreseen short lifespan. Timely diagnosis and proper therapy and the life that was once threatened gets rocket fuel enough to reach the stars.

Ana-Marija and Vera Miloshevska - "Embrace rarity"

Delayed development, affected coordination, breathing, lowered communication and cognitive skills are the characteristics of the RET syndrome, a disease that affects only the female gender. The colours in Ana-Marijas daily life are difficult to imagine. Our world, on the other hand, through her eyes seems a bit more predictable; uninterested, uninformed, cold; faces almost lifeless. What it needs to bring these two worlds together is an embrace, a look, and understanding.



Marko Noveski and Andrej Arsov - "Do Hurry up!"

Long and winding is the path of people with rare diseases through the labyrinths in which they fight for the right diagnosis for which they sometimes wait for several years. Andrej was diagnosed with Alagille syndrome, a disease that attacks the bile ducts, the liver, the heart and other organs, in the first two months after the symptoms occurred. He and Marko through the game they invented then and there and called it "Confusion" call for initiatives for equal success in the diagnostics of rare diseases.

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Iva Petrevska and Dani Dimitrovska - "A bell in a bubble"

A gentle touch, pressure, tiny injury or scratch is a threat to Iva's skin, as gentle as a bubble. The slightest daily childhood activities are the reason for painful sores on her body that require constant care and dressing; and very often the mucosa of internal organs is affected.

Presently there is no official therapy for Epidermolysis Bullosa.

Viktor Dimitrijoski and Bubo Karov - "Pancakes? No, thank you"

By his appearance and interests Viktor is no different from his peers, but he is different in something else. In his life he has never tasted chocolate, pancakes, ice-cream, walnuts, eggs, soy, meat or white bread. Since he was born he is on a special diet which is extremely expensive. The inability of his organism to process the intake of proteins in the usual way renders him nervous, angry, aggressive and scared of places with too many people and in advanced stages the disease phenylketonuria can bring to physical and mental disability.

Gjurgica Kaeva and Beni - "Blue Lips"

The oxygen is a luxury, as she says. The slightest physical effort, walking, climbing stairs, captures Gjurgica's breath and the blue lips are the first accompanying sign of this disease.

Pulmonary hypertension is a difficult, progressive disease which first endangers the work of the lungs and then the heart. It is a disease that can affect all ages and the treatment is very expensive. The disease does not choose an age, it can be congenital or acquired.

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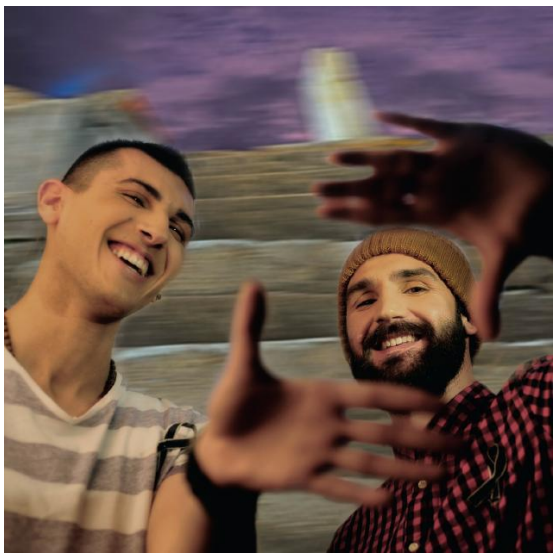
Dejan Angjeleski and Indira Kastratovik - "Challenge"

Swellings on different parts of the body make Dejan's daily life extremely complicated. The simplest movements can be extremely painful and the everyday activities impossible to perform. Some of the swellings can be life-threatening, especially those on the throat or the respiratory tract. The first signs of the disease HAE can occur during childhood and/or adolescence.
The medicine has not yet been discovered for this rare disease, for which there are treatment therapies, unfortunately none of them is available in Macedonia.

Pece, Marta and Viktorija - "Band"

Because of the FOP disease Viktorija's muscles turn into bones and now she is in a fixed position in her electronic wheelchair. Strumpell's syndrome, on the other hand causes muscle atrophy, and thus movement becomes more and more difficult for Marta. These lovely girls with typical teenage interests love music, are constantly on Facebook and Viber and enjoyed the make-up and singing for the joint melodious photo with Pece.



Slavche Shopovski and Toni Zen - "Trembling"

Affected coordination, trembling of hands and other uncontrolled movements are the consequences of Wilson's disease of which 15 people suffer in Macedonia, and the disease occurs between the ages 6 and 12. Slavche's organism instead of excreting the excess copper, which cells use for life functions, accumulates it in the liver creating free radicals, which cause problems for the other organs and the brain. He mustn't do sports and the medications he has to take are not on the positive list of medicines nor are they registered in the country.

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Vesna Aleksovska and Tanja Kokev - "Distance"

A slightly stronger embrace was enough reason for a broken bone or a bruise on her body. The lack of enzyme brings to the accumulation of Gaucher cells in the organs and in the bone marrow. Because of that the organs increase in size and the bones are prone to fracturing. Bruises, fatigue, faintness are all daily possible symptoms of the people with untreated Gaucher's disease. Vesna has been receiving therapy for five years now through donation, and her state is stable now so she leads a normal life.

Bojan Chunde and Rebeka - „1:10.000"

Hemophilia cannot be transmitted as cold because it is hereditary or occurs due to genetic change.

If Bojan gets hurt, his wound won't bleed faster but longer than usual because of the weak factor of coagulation of the protein responsible for coagulation of blood. The disease occurs at 1 to 10.000 people and is characterized with long external and internal bleedings.

In Macedonia there are around 300 registered persons with hemophilia.

Igor Parmakovski and Kristina Arnaudova - "I understand you!"

Pain, discomfort and problems with digestion of food are the main consequences that Igor feels facing Crohn's disease, which is manifested with inflammation and swelling of the digestive tract. Most of the symptoms of this disease attack the gastrointestinal system, from the mouth to the end of the large colon, but patients also report symptoms that affect the brain and the nervous system. It is believed the removal of the colon is the cure for the patients with ulcerous colitis. However, the surgical procedure is the "cure" only after all conventional methods of treatment fail to lead to improvement of the state.

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3. Research for rare diseases – April – December, 2015

In 2015 we implemented a research in the field of rare diseases in Macedonia in cooperation with Studiorum (Center for regional policy research and cooperation - http://www.studiorum.org.mk/). With this project we involved the patients, families, institutions and medical professionals.

The results of this research will give us the necessary recommendations and directions for improvement of quality of life of patients and families that face life with a rare disease. Main goal of this project is integration of social and health services for improvement of quality of life of patients through relevant data. The research will also give results about the needs of education of the medical professionals in terms of diagnosis and treatment of rare diseases.

Promotion of the study will be realized on Rare Disease Day 2016 so that we get more media attention and public and institution awareness.

Background

Despite having the characteristic of affecting small number of persons, in many cases, rare diseases are life-threatening and chronic diseases that tend to incapacitate the person suffering form it, and usually require long-term specialist care with quite high costs for the formal and informal, medical and non-medical care. The lack of diagnostics and effective treatments add to the factors that worsen life expectancy and quality of life for these patients.

There is a growing evidence and body of knowledge around the rare diseases diagnostics and treatment, as well as factors affecting their development or containment, which to a large extent assists the patients and the carers, as well as the community and the society at large. The medical sciences and the society have been successful at finding modalities for surviving and living with some diseases that were once almost certainly leading to death.

The activism around the issue of early diagnostics, treatment and care for persons with rare diseases have been also on a rise; there are many organisations and self0help groups that are actively involved in the policy making and putting the rare diseases higher on the political agenda, where the interest for addressing the rare diseases – from economic, societal and ethical point of view – is also growing, in the strive and commitment for providing basic human rights to all as equally and as equitably as possible.

In Macedonia, such political commitment and will has been recently expressed with the establishment of a state-funded Preventive programme for diagnostics and treatment of rare diseases; a fully-fledged intention to address the needs of persons with rare diseases, and to provide them with quality of their life to live in dignity, and where possible to contribute to the community and the society.

However, facing the limited possibilities and financial capacities, it is of outmost importance to provide evidence of the need – both by specific diseases as by specific treatments and other medical and non-medical forms of care, that can improve the life of the persons with rare diseases.

The Aim

Our aim is to address this gap of knowledge and information from the practice and experience, and to provide evidence that will inform the policies on the needs and priorities of these persons with regards to improvement of the mechanisms for early diagnosis and treatment; providing empirical and experiential information on for prioritization of the available resources – both material and non-material – to improved quality of life of RD patients, while addressing the social determinants of health.

The target groups that will benefit from such activities will include:

- RD patients
- RD patient caregivers
- RD patient organizations
- National and local authorities
- Experts in health economics, quality of life, planning and epidemiology

The Outcomes and benefits to the wider society

- Generation of data to feed into the recently established Registry of rare diseases in the country;
- Provide information on the quality of life and modalities for improvement of quality of life in multi-sectoral approach, involving other non-health sectors (social care, employment, education, etc);
- Will inform the policies on the size and scope, magnitude and severity of the conditions of the RD patients and their needs, so to be able to appropriately address them in the most feasible way;
- Will enable gathering information for health literacy of emerging cases of new RD patients and RD patient caregivers.

Given the impact of rare diseases on the quality of life of both patients and carers, it is likely that interest in its measurement will continue to increase among professionals, patients, and the general public. Improving the quality of life of people with rare diseases should be one of the most important goals of any health care intervention or multidisciplinary approach.

4. Building space for day therapy in the Children Clinic

In 2014 we were talking with the director of the Children Clinic about the space for receiving daily therapy (RD patients go to the clinic to receive therapy and usually there is no available beds because it is crowded). We agreed to make a special room in front of the hematology department (rearranging a part of the waiting space into a room for therapy).

5. Skopje marathon, May, 2015 - Running for better life! – Postponed Marathon for September because of situation in Macedonia

The Association of citizens for rare diseases Life With Challenges is continuing with the tradition to run at the Skopje Marathon in 2015.

On 10th of May, 60 participants from Skopje Night Running were prepared to run for Life With Challenges on 21 and 42 km. The marathon was postponed for September because of the political situation in the country.

This activity is for raising public awareness regarding rare diseases in Macedonia and about the challenges that people and families with rare disease face in their everyday life.

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6. Climbing Korab, 8th of September, 2015

This activity is contributing towards raising public awareness and it promotes healthy life style. In cooperation with the association for mountaineering Korab we placed our flag on the highest mountain top in R. Macedonia – Great Korab.

7. Educational activities, February – December, 2015

The Association of citizens for rare diseases Life With Challenges in cooperation with the preschool Detska Radost Skopje, started a series of lectures on the subject of rare diseases and special needs under the project called – Life is a puzzle, let's collect it together for the joy of the children. We are thankful that the municipality Gazi Baba supported this project and we hope that it will be a start of a larger project in the area of education and social inclusion. It was a pilot program intended as an incentive for education of the professionals involved in the education system. We hope to raise awareness about specific needs of families that face with rare diseases and special needs and to support discussion about appropriate inclusion of children with rare diseases and special needs in the schools in Macedonia.

Poster and flyer:

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The first workshop e was realized on 03.03.2015 by Nada Spandzevska on the subject of diagnostic and treatment of speech impediments in children, starting from communication in: receptive speech and expressive speech.

The goal of the workshop was: introducing parents with the work of the speech therapist (Detecting speech impediments in children, The way of work of the speech therapist) and introducing parents with the most often speech impediments

The second workshop was realized on 28.04.2015 on the subject of rare diseases and special needs by Vesna Aleksovska, patient representative. The goal of this workshop was introduction to the subject of rare diseases, the influence that they have on families and how to find ways to help and support them. The themes covered included the following subjects: What are rare diseases?, Rare diseases in EU and Macedonia?, Special needs of children with rare diseases?, How to support families that face life with a rare disease?, Discussion and questions.

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The third workshop was held on 12 May, 2015, on the subject of Stress in communication of rare diseases. The subject was focused on the caregivers as it is very important to take care for your own wellbeing while you are taking care of others. Especially when you are taking care of someone with a rare disease or a disability. When you take care of yourself you have more strength to take care of other in the long term. The health of caregivers has influence of everyone around. That is why this workshop was focused on different tools and ideas for reducing stress and improving wellbeing.

8. Organization of meetings for patients and families for different groups of rare diseases – March – December, 2015

As an association we encounter the needs of the patients to gather and talk to each other for specific diseases and challenges. These kind of gatherings help patients and families to support each other and communicate better with present medical professionals – directly connected with their specific disease.

Bulgaria – Macedonia Meeting, Kustendil, Bulgaria, 1-3 May, 2015

This meeting was focused on the following diseases: Familial amyloid polyneuropathy, Wilson Disease and Haemophilia. 15 patient representatives and doctors, from R. Macedonia were present at this meeting.

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Lectures:

- *Welcoming words* – Vladimir Tomov – Chairman of National Alliance of People with Rare Diseases Bulgaria
- Situation in Macedonia regarding rare diseases - Yesterday, Today and Tomorow - Vesna Aleksovska, Chairman of the association of citizens for rare diseases, Life With Challenges, Macedonia
- Balkan – Mediterranean clinical genetical profile of Familial amyloid polyneuropathy – Prof.Dr. Ivajlo Tarnev, Head of Neurological disease Clinic at Alexandrovska Hospital Sofia
- Wilson's disease and Niemann Pick disease type B – differential diagnosis and therapeutical strategies. Niemann Pick disease type C – clinical characteristics and treatment - Prof.Dr. Ivajlo Tarnev, Head of Neurological disease Clinic at Alexandrovska Hospital Sofia
- The new rare disease regulation - highlights - Prof.Dr. Rumen Stefanov, Dean of Public Health Faculty, Medical University Plovdiv
- Liver expression of Wilson's disease in Bulgaria – Prof.Dr. Lyudmila Mateva, National gastroenterological consultant, Head of Gastroenterology clinic, St. Ivan Rilski Hospital Sofia
- Porfirias in Bulgaria – Doc.Dr. Aneta Ivanova, Gastroenterology clinic, St. Ivan Rilski Hospital Sofia
- Opportunities for physical therapy in the rehabilitation of rare disease patients – Dr.Simeonova, Medical Centre "RareDis" for rehabilitation and training of people with rare diseases and their families, Plovdiv
- Hemofilia

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Regional Gaucher Meeting, Belgrade, Serbia, 8 – 10 May, 2015

The meeting was attended by almost 100 participants (including 27 Gaucher patients and 18 healthcare professionals) from Slovenia, Croatia, Bosnia and Herzegovina, Macedonia and Serbia.

Several interesting topics were presented, e.g. treating Gaucher disease, biomarkers, home therapy, results from clinical studies on eliglustat and taliglucerase alfa. Our special guest Dr. Mario Maas from Amsterdam gave us a lesson on the basics of MRI. During the whole meeting, there were many opportunities to meet each other and to network. We especially shared our feelings and opinions in three small sharing groups: for patients, for family members and for healthcare professionals.

On Saturday evening, following the Belgrade city tour, the 13th birthday of Serbian Gaucher Association was celebrated with gala dinner.

The meeting was overall a great success and it gave us hope to improve the Gaucher situation in our countries. As the situation is very different from country to country, learning from each other and sharing experience are very important.

We are grateful to Genzyme and Pfizer who supported the meeting.

The meeting was organised by:
Serbian Gaucher Association, Association of citizens for rare diseases “Life With Challenges”, Macedonia, Coalition of Associations in Healthcare in Bosnia and Herzegovina, Croatian Alliance for Rare Diseases and Slovenian Gaucher Association.

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Живот со Предизвици / Jeta me Sfida / Life With Challenges

Lectures and presentations at the meeting:

- Presenting current situation in participating countries (40 min)
- EGA's representative address (20 min)
- Prof. dr Nada Suvajdžić Vuković-Gaucher's disease – treating adult patients
- Doc. dr Maja Đorđević – Gaucher's disease – treating pediatric patients
- Dr. Mario Maas (Amsterdam) – Bone damage in Gauche r's patients
- Dr. Zorica Šumarac- Use of biomarkers in diagnosing and treating Gaucher's disease
- Therapy at home- Slovenian experience
- Dr. Zinaida Perić – Experiences regarding the application of Eliglustat (Cerdelga®)
- Dr. Milan Lakočević - Experiences regarding treating Gaucher's disease through clinical studies
- Workshops on: Living with Gaucher's disease (everyday life, position of society towards patients, quality of life)- group for patients, Living with a person suffering from Gaucher's disease- group for family members, Challenges and difficulties regarding treatment of Gaucher's disease- group for physicians and nurses.

Gaucher meeting Albania, 6-7 of September, 2015

Together with Tanya C.H. from EGA we met with patients and family members with Gaucher disease in Tirana at the University Hospital Center "Mother Teresa". This was the first ever meeting of Gaucher patients and we were honoured to be there. As a result of our meeting the patients and families are keen to set up their own patient group and the EGA will provide any support they need to do this. The patients and their family members are keen to work together to improve their quality of life, access to information and to work with doctors and the Ministry to achieve this. A meeting is being organised next spring to educate my doctors and bring all patients and families together and we have been invited to attend. Life With Challenges will organize families from Macedonia to meet with families in Albania.We hope one day the Albanian Gaucher group will be a member of the EGA, in the meantime we will work with them to help them achieve their goals.

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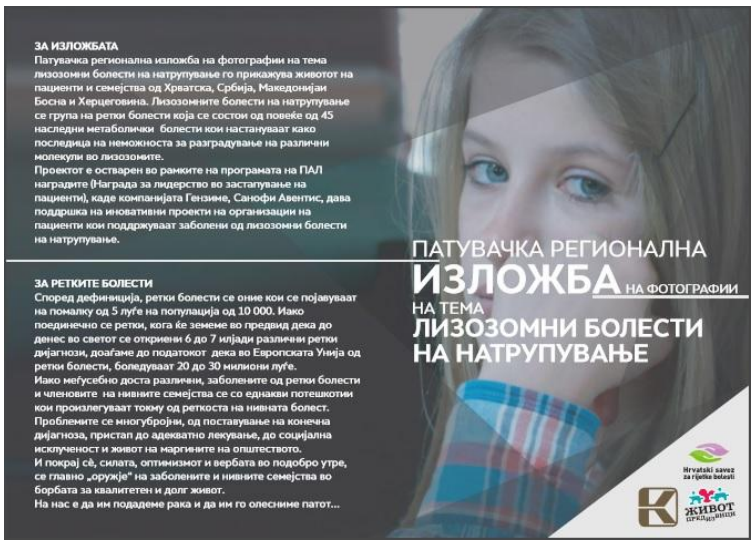
9. International Gaucher Day, 1 October, 2015

On 24.09.2015 at Kinoteka – Macedonia, we opened the First travelling regional exhibition of photos with subject – lysosomal storage diseases. This exhibition shows life of patients and their families from Croatia, Serbia, Macedonia and Bosnia and Herzegovina. Lysosomal storage diseases are group of rare diseases that is consisted from more than 45 hereditary metabolic diseases.



This project is realized in the frames of the PAL award program (Patient Advocacy Leadership Award), where Genzyme Sanofi Aventis is supporting innovative projects of patient organizations.

With this event, the Association of citizens for rare diseases Life With Challenges is also marking the International Gaucher Day (1st October). This day is marked in more than 40 countries, where member organizations of the European Gaucher Alliance, realize events for patients, families, doctors, researchers …

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From Gaucher Diseases, in Macedonia there are 8 patients. The disease is manifested in 3 types, but in Macedonia only Type 1 is present, and the symptoms are spleen and liver enlargement, fragile bones, thrombocytopenia, low hemoglobin levels etc … Until this year, 5 patients were treated on donation program by Genzyme Sanofi Aventis, and 1 through the Program for Rare diseases of the Ministry of Health in Macedonia. From this month, all patients have therapy provided by the Ministry of health. This therapy exists in the world since 1991. With this therapy all symptoms that occur in the years before treatment slowly disappear and the patients can have normal life.

10. South East European Meeting for Rare Diseases – R. Macedonia, 14 November, 2015

Rare Diseases in SEE speakers:

- **Mrs. Maja Ivanova, First Lady of the Republic of Macedonia, Honorary Patron of the Association of citizens for rare diseases "Life with Challenges"**
- **Mrs. Aspazija Sofijanova, Director, University Paediatric Clinic, Skopje, Macedonia**
- **Mrs. Vesna Aleksovska, President of "Life With Challenges", Skopje, Macedonia**
- **Momir Polenakovic, Velibor Tasic, Zoran Gucev, Skopje, Macedonia,** Rare diseases: an ongoing battle to curb the costs of diagnosis and treatment
- **Velibor Tasic, Skopje, Macedonia,** Steroid resistant nephrotic syndrome
- **Hadil Kathom, Sofia, Bulgaria**, Phenilketonuria
- **Stayko Sarafov, Sofia Bulgari**, Epidemiology, clinical features, differential diagnosis and treatment of TTR-FAP in Bulgaria
- **Ivailo Tournev, Sofia Bulgaria,** Selective screening, carriers testing and carriers follow-up program of the Bulgarian Neuromuscular Disease Society
- **Vukasin Andric, Zagreb, Croatia,** Pompe disease – recognizing and enzyme replacement therapy
- **Discussion**
- **Shire symposia, Gunter Harms, Berlin, Germany,** Why are orphan drugs different? Achieving sustainable access to treatment for rare disease patients
- **Gudrun Schlegel, Hamburg-Eppendorf, Germany ,** Mental retardation in PKU: The role of microglia
- **Stéphane Demotz, Lausanne Switzerland ,** GM1-gangliosidosis and Morquio disease type B

- **Eduard Pashke, Graz, Austria,** Recent developments in the laboratory diagnosis of MPS diseases
- **Zoran Gucev, Skopje, Macedonia ,** The Proteus Spectrum
- **Velibor Tasic, Skopje, Macedonia ,** Idiopathic infantile hypercalcemia
- **Venko Filipce, Skopje, Macedonia ,** Selective and super selective angiography of paediatric moyamoya disease angioarchitecture in the anterior and posterior circulation

11. Participation on conferences and workshops - January – December, 2015

Conferences and workshops help us in networking with national and international organizations (patients, doctors, researchers). It is also important for sharing information, education and building advocacy skills.

- ***EUPATI – European Patients' Academy, 2014 – 2015 training course***

EUPATI is a patient-centered team of 30 organizations, led by the European Patients' Forum, with partners from patient organizations (The European Genetic Alliance, the European AIDS Treatment Group, and EURORDIS), university and not-for-profit organisations expert in patient and public engagement, along with many European pharmaceutical companies. The common goal is to help patient advocates be more educated and involved in health care. The European Patients' Academy (EUPATI), will provide scientifically reliable, objective, comprehensive information on the research and development process of medicines. It will increase your capacity to be effective advocates and advisors, eg, in clinical trials, with regulatory authorities and in ethics committees. It also offers patients and the public basic information, training and education around health issues. The first training course is already happening with 50 participants from all around EU.

From Macedonia we have a participant from Life With Challenges. We hope to realize a basic training in Macedonia next year after the course ends and we translate materials in Macedonian language.

- ***Gaucher Leadership Forum, Berlin, Germany, 27 – 28 March, 2015***

The GLF is a scientific meeting sponsored by Genzyme that brings together approximately 200 participants from around the world, including physicians, academics, researchers and patient organization representatives to discuss current care for patients with Gaucher disease and the direction of future disease management. This year's GLF will be chaired by Timothy M. Cox, Professor of Medicine at the University of Cambridge. The theme for 2015 was: **"New Clinical Science of Gaucher Disease"** and it focused on the following objectives: Advancing therapeutics through greater understanding of pathogenesis (molecular & otherwise), Fostering collaborative and interdisciplinary research, Inspiring (recruiting) the next generation of clinician researchers and scientists.

- ***DIA EuroMeeting, Paris, France, 13 – 15 April, 2015***

The DIA EuroMeeting 2015 offers the ideal platform for healthcare innovators, patient advocates, decision makers and regulatory professionals to connect. They can network, scope potential new business partnerships and explore options for concrete solutions. This is why the EuroMeeting 2015 offers a significant return on investment for companies seeking to proactively manage a successful shift to a service oriented model. 13 patients were attending this conference through the fellowship grant program of DIA.

- ***Gaucher Expert Summit, Amsterdam, Netherlands, 17 – 18 April, 2015***

The Gaucher Expert Summit is an international meeting sponsored by Shire, which brings together, from around the world, healthcare professionals involved in the treatment and management of patients with Gaucher disease with the focus of "Driving Excellence in Patient Care". This educational event provides an opportunity to meet with other Gaucher physicians and to exchange views on best practice approaches to the management of patients with Gaucher disease.

- ***EURORDIS Membership Meeting, Madrid, Spain, 29 – 30 May, 2015***

This year's EURORDIS Membership Meeting (EMM) was abuzz with networking between over 250 participants who travelled from all over the world to Madrid. The EMM kicked off with the EURORDIS General Assembly, during which members approved theEURORDIS Activity/ Financial Report 2014 and the 2015 Action Plan & Budget. There was also a presentation of the 2015 – 2020 Strategy. Two representatives from member organisations of the EURORDIS Board were re-elected: Simona Bellagambi for UNIAMO, Italy and Avril Daly for Genetic & Rare Disorders Organisation, Ireland. The Board welcomed Françoise Salama as the new representative of the AFM-Téléthon, France and Nick Sireau, representing the AKU Society, UK. Meetings also took place around the EMM, including a Council of National Alliances Meeting, a RareConnectworkshop and the official launch and inaugural meeting of Rare Diseases International, the global voice for rare disease patients, which was attended by over 60 patient representatives from 30 countries.

- ***Partners for Progress: Patient advocacy in a changing environment, Lisbon, Portugal, 30 June – 1 July, 2015***

The Partners for Progress meeting sponsored by Celgene, brings together patients representatives from all over Europe, working in the field of haematological diseases. This year the meeting focused on the following subjects: Improving health in Europe – the innovative medicines initiative, advocacy in a changing environment (fundraising, creating patient information, governance for patient organizations), Ethics issues – patients' rights and responsibilities - can patient organizations partner with pharmaceutical industry without being influenced, Living with a long term illness – how it affects major life decisions, How can we ensure patient organizations are equipped to be effective advocates in the future.

- ***EEMEA-EEPO, Patient Organisations Connect, Istanbul, Turkey, 30 November – 1 December, 2015***

Concept of the Meeting EEMEA-EEPO meeting provides a unique opportunity for patient organisations in the Eastern Europe, Middle East and Africa region to meet colleagues active in the same or another disease area. Roche hosts EEPO(Experience Exchange for Patient Organisations) meeting in EEMEA region for the first time; helping patient organisations fulfil their chosen role by learning from each other's experience. The meeting's concept is Patient Organisations Connect. For patient organisations, it is crucial to connect with each other, governmental institutions, volunteers, pharma industry, international organisations and more importantly patients. Therefore, this meeting revolves around the concept of "connection/collaboration".

Thank you for your attention and I hope that we will continue our successful cooperation,

Vesna Aleksovska
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Information about the association:

- http://challenges.mk/
- https://www.youtube.com/user/lifewithchallenges
- https://www.facebook.com/LifeWithChallengesi
- https://www.facebook.com/groups/312483895490987/
- https://twitter.com/ZivotPredizvici

Life With Challenges is active member of: