Живот со Предизвици / Jeta me Sfida / Life With Challenges

Dear members and supporters,

The Association of citizens for rare diseases "Life With Challenges" is continuing its work in the field of rare diseases. In 2016 we 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 Alliance for patient organizations – APO in Macedonia, an organization that is working on helping patients and families that face cancer. Our focus as a rare disease organization in this alliance is on rare cancers as this is a field that still needs improvement in Macedonia as these patients have no access to innovative therapies yet.

For our program in 2016 we received donations from different private companies in Macedonia, than donations from pharmaceutical companies such as Genzyme-Sanofi Aventis, Celgene International, Hofman La Roche, Pfizer, Biomoarin.

For all activities, there is information on our web page, facebook and youtube:

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

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

Vesna Aleksovska,

President of the Association of citizens for rare diseases,
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Life With Challenges is member of:

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Activity report for 2016

In 2016 "Life With Challenges" implemented many activities for raising public awareness, strengthening patients, cooperation and networking with other organizations on national and international level and communication with the health institutions in Macedonia.

As in 2015 the ministry of health is financing the Program for Rare Diseases with additional fund from cigarettes tax and we are already aware that 21 new medicines are tendered and supplied to patients with 12 different diseases, our activities will be focused on unmet needs of patients. In 2016 the Ministry provided more than 21 different drugs for more than 12 rare diseases and we hope that this program for rare diseases will continue to grow as there are more and more patients registered in the register for rare diseases at the Ministry of Helath.

We are aware that many groups of rare diseases are still not included in the list we will focus our activities on those diseases as our mission is to advocate for provision of high quality care for all patients with rare diseases.

The New Programme of the Ministry of Health for 2016 is as it follows:

1. Diseases which can be treated with medicines;

2. Diseases which are rare, but for which there is no treatment but which are important for the diagnostic and planning.

In the registry of patients with rare diseases are included solely the diseases listed in the ORPHAN list of rare disease – 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 Macedonia Medicines are provided or 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, and 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 is expected that in 2016 total funds in the amount of 203.000.000,00 denars will 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 hope that changes will be made in the program for 2017 since as patients organizations we all reacted to the limitation of the number of patients (20 per diagnosis). 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.

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1. Rare Disease Day, 29th of February, 2016

For rare disease day "Life With Challenges" is planning an event following the moto set by EURORDIS where the organization is a full member since 2013. This year "Life With Challenges" planned and organized the event together with 13 rare disease organizations, members of the National Alliance for Rare Diseases founded in May, 2014. Together we realized a press conference with debate, where participants were representatives of relevant institutions, patients, families and doctors, open for the public audience and media.

The Managing Director of the Agency of Medicines and Medical Devices Ms. Marija Darkovska Serafimovska discussed the importance of ORPHA medicines. Although the rare diseases are few and generally the medicines necessary for them are not interesting for the pharmacological industry because the research they require is expensive and the market is small, lately there has been some positive advance as a result of sponsored research by the governments, shorter administrative procedure and the patent for these medicines lasts longer. In regard to these medicines in Macedonia in 2015 a continuous supply was provided and this was thanks to the amendments in the Law on Excise Tax and the work of the Rare Disease Commission. The Agency for Medicines and Medical Devices will continue to give its support in the same and similar way.

The Chairperson of the Commission for Rare Diseases PhD Aspazija Sofijanova, MD pointed out that living with a rare disease is a challenge; she also stated that it is an even greater challenge to maintain the disease under control. She emphasized that the rare disease registry is regularly updated and that we should all work together on greater integration of the people with rare disease in our society. She said she was grateful to everyone who is part of this struggle, as well as the media for the positive coverage of this issue. Ms. Sofijanova underlined that it is not only therapy that matters but also the family and the integration in society.

The MANU Representative, PhD Dijana Plasheska Karanfilska briefly stated how important is diagnostic and that although it is expensive it exists. Modern diagnostic tools are available to patients in Macedonia and with these tools prenatal and genetic diagnosis can be carried out, which help to determine the possibilities of a rare disease occurring in the family.

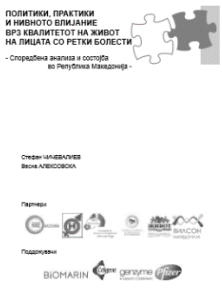
The President of the National Alliance of Rare Diseases, Vesna Aleksovska stated "At the beginning, in 2009, I was a lone fighter patient, but today we have a big family where we all fight the battle of Rare Diseases." She summed up how important treatment was and pointed out the research that Life with Challenges carried out in cooperation with "Studiorum".

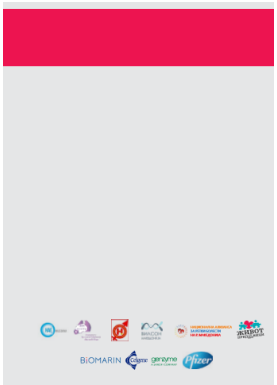
Presentation of the research on rare diseases by Stefan Chichivaliev (Studiorum)

Stefan Chichivaliev discussed the road which a rare disease patient must take. This road begins with a diagnosis which can be very prompt or it can take longer. The second part of that road is actually the most important part which unifies the primary, secondary and tertiary health care and which helps the patient to maintain a stable stat with an appropriate treatment, which is actually the last part of the road. Without appropriate support the families fighting a rare disease face huge problems, foremost from an economical aspect as well as a social aspect.

There was also a public event for raising awareness where we shared promotional materials (flyers, posters, brochures) to the public and the media.

The part of "Life With Challenges" in Rare Disease Day, 2016 was a promotion of the study for rare diseases that was realized in 2015 and can be downloaded at the following link - .





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Also we participated in the “Hug For Rare” campaign with the “Croatian alliance for Rare Diseases” and the “National Organization for Rare Diseases Serbia” in the effort to accomplish a Guinness record in hugs for Rare Disease Day.

This year we will also organized an event in **Ohrid** with the help of our members and volunteers, with special thanks to Gordana Loleska, mother of a child with Alport Syndrome, and to many private companies from Ohrid who supported the event. The Organisation of the Red Cross in Ohrid together with the Association for cycling, mounteneering and Eco-culture Z’s Avantura, on the 27th under the title HUGGING OF GALICHICA organized a hiking tour on the mountain Galichica. The pupils from the State Secondary School “Sv Kliment Ohridski” marked the rare disease day by running an athletic cross race which took place on the 29.02.2016 at the Ohrid Lake Quay.

In Prilep volunteers with the support of the Municipality of Prilep organized a fashion show in the cinema Miss Ston on the 27.02. 2016, with special thanks to Jasminka Hristoska a patient with acromegaly, who organized this event. Also we had a public event in **Bitola** with the help of our partner from the Alliance of Patient Organizations – Hepar Center Bitola.

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As a continuation from rare disease day, we also were involved in realization of a lecture in April for doctors in the Medical Faculty in Skopje, Macedonia.

2. Skopje marathon, 8th of May, 2016 – Skopje Night Running runs for Rare Diseases!

The association of citizens for rare diseases LIFE WITH CHALLENGES continued with the tradition to participate on Skopje Marathon. This year the association Moment Plus from Gevgelija was also participating in the marathon.

On 8th of May, 2016, 60 participants from Skopje Night Running run for Life With Challenges on 21 and 42 kilometres, and also there will be additional participants running for Moment Plus on 5 kilometres. The voice of rare diseases is stronger when we are together.

This activity is for raising public awareness about rare diseases in Macedonia, emphasizing the challenges that people with rare diseases and their families face in every day life. We are thankful to all that support us until today and we will continue with the cooperation further. We hope to contribute for a better future for people with rare diseases as an association.

3. Press conference for emphasizing problems of people with rare diseases that need urgent solutions

The National Alliance for Rare Diseases of R. Macedonia, with the support of its members, organized a press conference to inform the public and the institutions that there are some problems that need urgent solutions. As patients are thankful for the progress in the treatment of rare diseases, there are still many things to be done for improvement of life of patients with rare diseases. Special thanks to the organization ESE for organizing the conference.

4. Climbing Korab, 4th of September, 2016

The Citizens' Association for Rare Diseases Life with Challenges continues the tradition to participate in climbing Macedonia's highest mountain top Golem Korab in 2016. On the occasion of 8th of September the traditional climbing of Golem Korab тоок place on the 4th of September, Sunday. ZPK Korab pitched the flag of the Citizens' Association for Rare Diseases Life with Challenges on the mountain top.

5. Climbing the highest mountain in Western Europe – Mont Blank in France in support for rare diseases in Macedonia

Ohrid citizens strike again with support for people suffering from rare diseases and kidney diseases in Macedonia. The highest peak in the Alps amd thus the highest mountain in Western Europe- Mont Blank in France was succesfully climbed in the period from 19.07 to 26.07. This expedition was led by renowned macedonian mountaineer Misko Taneski together with members Konstantin Gorgievski and Vlatko Karbaleski. It took 4 days to climb this peak of 4180 meters. This way Macedonian mountaineers joined the large number of athletes who support people with rare diseases and kidney diseases with ultimate strong message that THEY ARE NOT ALONE AND THEY ARE NOT INVISIBLE! Special thanks to Gordana Loleska who initiated this cooperation with the expedition. We will continue this cooperation next year also.

6. Organization of a patient meeting for Gaucher families and doctors from Albania and Macedonia, 8-9 October, 2016

To mark the International Gaucher Day, the Macedonian association for rare diseases LIFE WITH CHALLENGES together with the Albanian association organized a meeting on 8-9 October in Struga, Macedonia. At the meeting we had families, patients, doctors and nurses connected to Gaucher disease type 1,2 and 3. Also we had representatives from Genzyme and Pfizer who sponsored this meeting.

Patients talked about their challenges and problems regarding therapy and they shared experiences about what is going on in their countries at the moment, how they were diagnosed, what kind of therapy do they receive, and what kind of tests do they do ... they talked about home treatment and will it be a good option to have in future. We have the possibility to also hear a story from a patient who was on a clinical trial on Cerdelga and now he is still on the pill, which was a new experience for all patients. We truly hope for a future where patients together with doctors can choose therapy that is most suitable for the patient and with most quality of life for the patient and the family.

Doctors from both countries (Dr Biljana Coneska Jovanova) had presentations about what is Gaucher disease, and about the patients in the countries (treatment, challenges, future options). We realized that we face some same and some different problems and that we can help each other to do more for the patients.

The support from EGA is very important, because of EGA we manage to came together and talk, and now there is an association of patients in Albania that will soon join EGA. We agreed that the most important thing in future that we expect from EGA in this region is education of doctors about diagnosis and treatment. We hope to have the next meeting in Albania in spring time and to have guest lecturers - doctors from EU.

7. **First International congress on persons with special needs – And why not be different?, 14-16 October, 2016, Skopje, Macedonia**

At this congress representatives from "Life with challenges" were participants – lecturers, presenting the study on rare disease - *Study of policies for the health and welfare of people with rare diseases in the Republic of Macedonia.*

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8. Round table on rare cancers, 28th of October, 2016, Skopje, Macedonia

The Citizen Association for Rare Diseases "Life with Challenges" organized a round table on the topic of rare cancers in Macedonia at Best Western Hotel in Skopje on October, 28th , 2016. This activity was about raising the institutional and public awareness regarding the inaccessibility to treatment for patients suffering from rare forms of cancer for which at the moment no therapy is procured neither through the FHIM nor through the rare disease programme of the Ministry of Health nor through the oncology and haematology clinics.

One of these rare cancers is the Myelodysplastic Syndrome and the day of MDS is marked in the month of October. One MDS patient is already receiving treatment through donation and another one received a similar donation in order to stabilize the state before starting bone marrow transplantation. There are different types of MDS and they fall into the rare disease group and there are the so called orphan drugs for their treatment. Unfortunately, at the moment we do not have precise information about the number of patients in need of treatment because of the different classification of MDS, however, the probability is that around ten patients need innovative therapy which will suppress the disease progress and improve patients' lives.

Also there are many other different types of rare cancers such as: multiple myeloma, malign myeloma, gastrointestinal stromal tumors, gastroenteropancreatic neuroendocrine tumors, acute myeloma leukemia, angiosarcoma, neuroblastoma and many others; some of these have been publicized as rare in the Rare Disease Programme back in 2009, yet today they are not included in the Rare Disease Programme. We believe that they should not be discriminated as such and that these patients should receive the necessary treatment as they in fact do so in our neighbouring countries such as: Bulgaria, Croatia, Slovenia, Montenegro and Serbia made progress in this area.

Statement Vesna Aleksovska, President of "Life with Challenges"

"Rare diseases were invisible for a very long time in Macedonia. Since 2015 and the commencement of the Rare Disease Registry and the increased finances in the Rare Disease programmme finaly ofer 13 diagnosis, that is 150 patients have received treatment. Until then these patients did not receive any type of medication and were left to their own means. Unfortunately, the patients with rare forms of cancer who are at the hematology clinic and oncology clinic still do not receive the necessary medicines. As a patients' association we believe that there is discrimination towards these patients because they are faced with a rare disease and should be provided with medicines that will prolong

their life, as well as improve their quality of life. One of these rare cancers is myelodysplastic syndrome (MDS) which is marked in October and for which we have created a brochure aimed at patients facing with this disease. One patient from Macedonia with MDS already receives the necessary treatment through a donation. We believe that treatment should be provided for all patients with rare cancers who are registered at the clinics. Unfortunately we do not have the exact number at the moment, but such diagnosis are rare and can be found at the Orphanet list; and at the moment this is one of the criteria for procurement of medicines for rare diseases. The criteria of 20 patients per diagnosis is discriminatory and as an Association and Alliance for rare diseases we reacted to the number 20 because we fear that the number is very limiting and there is uncertainty what will happen when the 21st patient is registered. We appeal for the rare disease programme to additionally expand financially, that is to say, for the amount that had been promised at the beginning (twice more than the present amount of 3 million euros) so that more patients receive the necessary medicines. Innovative medicines used in the world should be used in Macedonia in order to prolong and improve the patients' lives.

Statement by patient with MDS –Dragi Lazevski

"In September I felt as if without energy or fitness, after all the tests the diagnosis of megaloblast anemia was confirmed in November. I was in dire financial situation and unable to provide the necessary medicines. Fortunately through doctor Irina from hematology, whom I am deeply grateful for all her endeavours and those of the nurses and staff at the hematology clinic, I received treatment through donation from the company Celgene. After the treatment I felt wonderful and I can work and spend time with my family and do everything that I did before the disease. I must say that is important that innovative therapy should be available to al patients in order to have good quality of life and to prolong their life. No family alone can afford to provide treatment. Financially it is terribly difficult."

Statement – Biba Dodeva, President APO

"In this pre-election times citizens need more than slogans. Therefore I publicly call upon all public institutions, present and future government, present and future health managers, I call upon the politicians and all those which our health depends on to put cancer as a priority in the health programmes and to understand the necessity of URGENT implementation of the National Strategy for Controlling Cancer in Macedonia that will enable and engender joint detection of problems and a comprehensive approach for surpassing them and resolving them.

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Statement- Davor Duboka, Executive Director of the National Rare Diseases Organisation of Serbia
"I would like to point out the importance of what has been happening in Serbia recently, namely the HIF is already in negotiations with the pharmaceutical companies to create a priority list of innovative medicines that will be procured through a special fund in order to help patients facing difficult diseases, not only rare disease, but also cancer and other diseases requiring innovative therapies. In Serbia all the associations have come together in our demands for innovative medicines and we have finally reached and understanding from the institutions for the needs of the patients. For the time being 20 new medicines will be procured through the list of priority innovative medicines."

Statement – Borislav Gjuric, President of the Rare Disease Association in Bosni and Herzegovina
"Bosnia and Herzegovina are unfortunately in a bad political situation because of all the problems we were facing in the past and have been facing ever since. The separation into cantons is very complicated and in some regions the situation with medicines is very good while in others it is devastating. Macedonia has a simpler political system and it is really incomprehensible why there is not a solution for the cancer patients yet. Personally I think it is literally manslaughter not to provide medicines for a patient whose life could be spared. It is not manslaughter just to kill someone. Health is a basic human right and no citizen should be deprived of it. All countries in the region make efforts to improve the quality of life of patients; we collaborate with all the associations and support regionally each other and we will not give up from the basic human right of life – health. We cooperate with institutions and media as well… one by one we might be few, but together we are many and together our voice is stronger and reaches further."

9. The first regional conference on Wilson's disease, 25-16 November, 2016 Skopje, Macedonia

The first regional conference on Wilson's disease organized in Macedonia. Patients and medical professionals from Bulgaria, Serbia and Macedonia spoke at the conference, as well as prof. Peter Ferenci from AKH, Vienna, leading expert in Wilson's disease.


10. Organizing FOP day in Valandovo, Macedonia, 3rd December, 2016

On 3rd of December, our member Viktorija Penovska with the help of her school and her friends and family organized an event in her school focusing on the importance on accepting disability and the importance of social inclusion.


11. Postal stamp for Rare Disease Day

Last year a member of the association for rare diseases Life with challenges, a mother with a child with Alport syndrome, Gordana Loleska, employed in 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 Macedonia. This initiative was supported by many medical professionals in Macedonia who send 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. This year the post accepted this initiative and we hope that the stamp will be ready for distribution for rare disease day 2017. The name of the stamp will be Children with rare disease.

The stamp will be promoted by the national alliance fir rare diseases of Macedonia and also by the post office. It is a great idea that came true … a nice way to promote awareness.. we hope that other countries will do this also.

We are very proud of Gordana Loleska who enthusiastically took this idea forward. We also want to say thank you 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. Macedonia, with its president Ms. Vasilevska Sonja.

12. Rare Disease Day Flag under water for New Year, 25 December, 2016, Ohrid, Macedonia

For the 15th time, the members of the Dicing center Amfora, and the Club for under water activities Ohrid, in cooperation with the Alliance for under water activities of Macedonia, decorated the Christmas tree under water in Ohrid lake, Macedonia. Traditionally as every year they decorated the tree in the lake at the Bay of Bones. Even the youngest members of the diving club participated in this activity.

This year the Ohrid divers dedicated the decoration to the children with rare diseases in Macedonia. Goran Balevski said that this year they decided to sent a message to the public for support of the children with rare diseases.

This cooperation was made possible with the efforts of our member Gordana Loleska from Ohrid.

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13. Participation on conferences, workshops and training - January – December, 2016

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

- **EURORDIS meeting in Edinburgh, Scotland – 25 – 29 May, 2016**

The first plenary focused on what has been achieved so far in the field of rare diseases its recognition, impact, access to treatment and enabling patient to have good quality of life. ECRD has become a platform for sharing experiences and advancement. Among other things what was pointed out were the goals achieved such as EUPATI -education for patients. What followed was an address by Tom Shakespeare who gave a memorable speech about disability. Disabled by society was the theme of his speech. He talked about the importance of a more holistic approach to disability where there would be better solutions in terms of education and social care. The most important point was the CRPD- UN Charter on Rights of People with Disabilities. This charter has already been adopted by 164 countries and it vouches equality and respect for people with disabilities. The three main points this charter addresses are: Keep science and drugs sustainable and at reasonable cost, Multifactorial approach to disability (Psychological, Sociological), People with disabilities (including RD patients) hold governments to account with the CRPD. The following session was dedicated to patients and it started with a lovely and encouraging presentation by Igor Ban. Igor told the audience his story how he fought a rare form of blood cancer as a child. Nowadays Igor continues to work for patients with rare diseases working as web content manager for RareConnect. The panel discussion of patient representatives focused on how new technologies and social media help patients connect world-wide. The patients were unanimous in the conclusion that RD are a global priority and that patients are part of the progress.

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Our representative attended the sessions relevant to our work where various issues were discussed and different cases were presented. In one of the sessions our representative attended the focus was on the set of recommendations aimed at advising Member States and the European Commission on issues that should be considered when organising the holistic care of RD patients within national health and social system. These recommendations have only just been adopted by the Commission Expert Group on Rare Diseases. Mr. Jaroslaw Waligora from DG Health talked about the new EU Recommendations which include the CRPD and also emphasised the need for a more holistic approach to the treatment of rare diseases. Health is a state of complete physical, mental and social well-being. Mr. Eduardo Tizziano, Director of the Clinical and Molecular Genetics, Rare Disease Unit, Hospital University Valle Hebron, VHIR, Barcelona, Spain, shared the experience on how RD patients are treated in this hospital. Namely, he discussed the importance of the triangle between the patient, the family and the caregiver. He emphasised the need that the patient no longer belongs to the hospital it is important to keep the patient at home surrounded by family and leading a fulfilling life. Today this can be done thanks to the advancement of technology. Regarding rare diseases the patient no longer has to travel from one specialist to another, in fact, the innovation that Mr. Tizziano offered was that doctors should come to the patients and each RD patient is assigned a case manager who manages the other specialists and all the other involved in the patient's care. The major points he mentioned were: Coordinator, Faculty member (specialist), Case manager, Psychologist, Administrative support, Trained social worker, Capacity to integrate all.

The main focus in the sessions was integrated care and the necessity of a holistic approach to treatment and improvement of quality of life. Other topics which attention was paid to were prenatal care as well as changes in legislation in order to enable patients to have greater access to treatment and care. The latter referred to hemophilia patients.

In one of the sessions titled 'Patients becoming People' it was underlined that better care and access to treatment to chronically ill patients can render them active members of society and thus have a beneficial effect on both patients and community. At the moment some of the governing policies are obstacles and many chronically ill patients are stuck in the system.

The last panel session emphasised the need to adapt the global to the local and the importance to understand the cultural setting and how to approach people. A lot has been done through National Alliances and even more needs to be done so that RD can become truly international and to put the voices of people to voice the need of the community and to empower communities to share and use new knowledge and technologies. Holistic approach supported by the UN, UNESCO, IMF and WHO is of utmost importance regarding RD both for improved treatment and quality of life as well as for changing and removing the stigma of RD patients. National and international debates are more than necessary to achieve a brighter future in the field of RD.

- **EGA meeting in Zaragoza, Spain – 28 June – 2 July**

The EGA is delighted to announce that during its Biennial General Meeting in Zaragoza on 29 June, its members expressed their wish to amend the founding constitution by abolishing the differentiation between full and associate membership. With a voting result of 69% in favour of providing full rights to non-European member associations, the majority of the members expressed their clear wish that the EGA becomes a global group.

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On 29 June, Pascal Niemeyer was elected by the new board of the European Gaucher Alliance as chairman. Pascal has been working on the board since 2008 and is responsible for the areas of public relations and liaison with the EWGGD. Pascal takes over from Jeremy Manuel, who decided to step down as chairman to make way for the next generation, whilst assuring his dedication to continue to support the Gaucher community by agreeing to remain on the board.

The entire board would like to thank Jeremy for his tireless engagement in building and developing the EGA for more than 20 years.

The new board was formally announced as: Vesna Aleksovska (Macedonia), Biljana Jovanovic (Serbia), Anne-Grethe Lauridsen (Denmark), Jeremy Manuel (UK); Pascal Niemeyer, Chair (Germany), Johanna Parkkinen (Finland), Jasenka Wagner (Croatia), Sandra Zariņa (Latvia), Irena Žnidar (Slovenia).

Some very interesting discussions took place during the biennial meeting. Workshops where participants were split into four groups facilitated by board members. To inspire the discussion a short introduction was given on possible subjects to discuss, based on the information given in your Country Reports which were sent in ahead of the meeting.

The board has now evaluated all the written feedback from each groups and split the issues raised into the following 3 areas:

- How to get a diagnosis, switching treatment, treatment or no treatment, social aspects of GD
- Running an association; why and how
- Creating awareness towards different target groups

Looking deeper into the feedback, the overall theme identified was "Awareness" and so it was decided that a small group within the board should look at what this means on different levels. Based on this work the group will identify possible new projects to propose to the full board at the next meeting in January.

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- **Partners for Progress meeting, Vienna, Austria, 4-6 July, 2016**

Workshop on Effective advocacy for the future. Learning from the reality of living with an illness and about the role that patient organizations play in supporting people living with a serious illness. It provided an opportunity to share experience with other patient organizations and with extended range of participants there were new ideas and opportunities.

- **Participation in a Balkan meeting of organizations on a workshop in Belgrade, Serbia, 6-8 September, 2016 – subject - 'Incorporating the patient voice into health technology assessment'**

Access to medicines in Europe is becoming increasingly dependent on cost considerations using health technology assessment and it is essential that the patient perspective is taken into account in these deliberations. What is the reality of living with an illness? How can a new treatment make it easier to cope with the illness? These are the sorts of questions that only people directly affected by the illness can answer and patient organizations can provide evidence on how a new medicine is valued by patients and their caregivers.

Association of citizens for rare diseases "Life With Challenges – Bitola
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- **FIFTH MEETING OF SEE FOR RARE DISEASES, ON 12TH NOVEMBER, 2016**

The Macedonian Academy for science and art had the opportunity to organize the fifth meeting of SEE for rare diseases, on 12th November, 2016.

Int he organizational committee of this meeting are: Momir Polenakovic, President, Zoran Gucev, Secretary, Nada Pop-Jordanova, Ilija Filipche, Zivko Popov, Velibor Tasic,Aspazija Sofijanova, Vesna Aleksovska, Dijana Plaseska Karanfilska


The program was as it follows:

09.15-09.30 **Stefan Chichevaliev, Vesna Aleksovska, Skopje Macedonia**, Policies, practices and their impact on the quality of life of people with rare diseases

09.30–09.50 **Momir Polenakovic, Skopje, Macedonia**, Rare genetic kidney diseases

09.50-10.15 **Milosevic Danko, Zagreb, Croatia**, Atypical hemolytic-uremic syndrome (aHUS) in Croatian children

10.15-10.40 **Zoltan Prohaszka, Hungary**, Overview of complement deficiencies

10.40-11.10 **Milan Lakocevic, Belgrade, Serbia**, Enzyme Repleacement treatment: Serbian experience with taliglucerase alfa

11.10-11.40 **Adrijan Sarajlija, Belgrade, Serbia**, Glycogen storage diseases – single center experience

11.55-12.20 **Johannes A. Mayr, Salzburg, Austria,** Mitochondrial diseases – Are they rare or common?

12.20-12.45 **Velibor Tasic, Skopje, Macedonia**, Pitt Hopkins syndrome an update

12.45-13.10 **Zoran Gucev, Skopje, Macedonia**, The spectrum of growth disorders: the Macedonian part

13.10-13.35 **Rossella Parini, Milan, Italy,** Mucopolisacharidosis: diagnosis and treatments

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Session II - Moderators: Ilija Filipce, Velibor Tasic, Zivko Popov

14.10-14.40 **Hans Joachim Seitz, Hamburg, Germany**, Endocrine Disruption as cause of (rare) diseases

14.40-15.10 **Oliver Bartsch, Mainz, Germany,** Next generation sequencing in pediatrics (in Germany)

15.10-15.35 **Jan Halbritter, Leipzig, Germany**, Mutation analysis in kidney stone disease

15.50-16.05 **Zlate Stojanovski, Skopje, Macedonia**, Polycythemia rubra vera in patient with Gaucher disease

16.05-16.25 **Liljana Saranac, Nish, Serbia,** Microprolactinoma in childhood and adolescence; a rare or underestimated disease

16.25-16.45 **Zoran Gucev Skopje, Macedonia**, M.Gaucher: treatment

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

Vesna Aleksovska,
President of the Association of citizens for rare diseases,
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Life With Challenges is active member of:

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