

REPORT

"THE CHALLENGES OF THE POST-PANDEMIC EUROPE IN THE HEALTHCARE SECTOR"

FROM THE CONFERENCE:

A prescription for the future of Europe in the face of social, economic, and health challenges after COVID-19. European Union, member states, civil society.

DATE

24 November 2021

VENUE

Hotel Victoria Sofitel
in Warsaw

Live transmission

About the foundation

Institute for Social Policy Development (IRSSO) was established in 2020. It is a civic think-tank created under the Kulski Foundation for Polish-American relations. IRSS's mission is to provide decision-makers with cross-cutting analytical material by engaging a wide range of opinion leaders, sector experts, representatives of the social side (employees, employers, etc.), as well as the media and representatives of the public administration – both at the central and local government levels – in public discourse.

Conference idea

The conference of the Kulski Foundation and the Institute of Social Policy Development perfectly fits into the search for a new way for a united Europe, which began with the Future of Europe initiative. This is a unique opportunity for EU citizens to be heard by EU decision-making bodies. Member States face many challenges related to the redefinition of European identity, social and economic problems in the dimension of both the entire continent and nation-states, as well as the need to use social capital and the potential of civil society. To the need of the search for optimal solutions to the challenges outlined above is also added the awareness of the EU institutions per se that MS and their citizens should have a greater influence on shaping a united Europe in the future. The COVID-19 pandemic and its consequences set a particular context to look for new ways of developing the European Union. The scale of the problems that individual member states have faced clearly indicates the need to tighten cooperation within our continent. The strategic security of Europe depends on it from the perspective of future generations. The crisis that the whole Europe faced as a result of the pandemic has left its mark on many life areas both at individual, family, social, as well as national, and regional levels. The new reality forces us to redefine our priorities in terms of economic, social, and, first of all, health security. As the experience of the last 1.5 years has demonstrated, the general condition of individuals and entire societies and economic systems depends on health safety and security. Future Europe must be more resilient to crises and health threats that are inevitable in an increasingly globalized world. Health security has currently become the foundation of security in every other field.

Europe and its citizens, among others

- should tackle the upcoming oncological tsunami,
- should find an effective response to growing civilization diseases,
- have to deal with the consequences of the pandemic for the mental health of individuals, especially children, and adolescents,
- must not forget about the solidarity with those, who are most in need of support – in particular, people suffering from rare diseases and seniors,
- must seek the best solutions to ensure safe access to plasma-based therapies, the collection of which is particularly at risk in the face of phenomena, such as pandemics,

- should use and benefit from the wide opportunities for the exchange of knowledge, information and scientific resources, not only within the European community but also in the transatlantic dimension,
- should expand international cooperation at the level of non-governmental organizations and public institutions to exchange experience and know-how and support the development of bottom-up initiatives.

The future of coming generations depends on how Europe and Europeans will respond to the challenges mentioned above.

ORGANIZERS:



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Conference
on the **Future**
of **Europe**

**A prescription for the future of Europe in the face of social,
economic, and health challenges after COVID-19.
European Union, member states, civil society..**

BLOK II – “THE CHALLENGES OF THE POST-PANDEMIC EUROPE IN THE HEALTHCARE SECTOR”

PANEL IV: Civilization diseases – scale, determinants and threats vs. treatment opportunities.

Moderator: Bartosz Kwiatek, Polsat News journalist

Keynote speech: Maciej Miłkowski, Undersecretary of State, Ministry of Health

Panelists:

1. **dr Tomasz Latos**, Chairman of the Polish Parliamentary (Sejm) Health Committee
2. **dr n. med. Roman Topór-Mądry**, President of the Agency for Health Technology Assessment and Tariffs
3. **prof. Paweł Śliwiński**, President of the Board of Polish Society of Lung Diseases
4. **prof. Brygida Kwiatkowska**, National consultant in the field of rheumatology
5. **prof. Robert Gil**, Head of the Department of Invasive Cardiology of the Clinical Hospital of the Ministry of the Interior and Administration (MSWiA) in Warsaw
6. **prof. Teresa Jackowska**, National Consultant in the field of pediatrics
7. **prof. Leszek Czupryniak**, Head of the Diabetology and Internal Diseases Clinic, Medical University of Warsaw
8. **prof. Jarosław Fedorowski**, President of the Polish Federation of Hospitals, President of the Polish Coordinated Healthcare Society
9. **dr hab. Adam Maciejczyk**, Chairman of the Polish Oncological Society
10. **dr Małgorzata Gałązka-Sobotka**, Vice-President of National Health Fund Council, Lazarski University

PANEL V: European solidarity and the diversity of its dimensions – rare diseases and their treatment as an element of the civilizational and ethical foundation of modern Europe .

Moderator: dr Jakub Gierczyński, Healthcare system expert, member of the Council of Experts adjunct to the Commissioner of Patients' Rights

Keynote speech: prof. Krystyna Chrzanowska, Head of the Department of Medical Genetics at the Children's Memorial Health Institute in Warsaw, regional consultant in the field of clinical genetics

Panelists:

1. **Maciej Miłkowski**, Undersecretary of State, Ministry of Health
2. **Grzegorz Błazewicz**, Deputy Commissioner for Patients' Rights
3. **Barbara Dziuk**, Chairman of the Parliamentary Team for Rare Diseases
4. **prof. Anna Latos-Bieleńska**, Head of the Department of Medical Genetics, Medical University in Poznan

5. **dr hab. Anna Szmigielska-Kapton**, Department of Hematology, Medical University of Lodz
6. **dr Tymoteusz Zych**, Vice President of the Management Board of Polish Non-Governmental Initiatives Confederacy, Member of the European Economic and Social Committee
7. **Stanisław Maćkowiak**, President of the Federation of Polish Patients
8. **Krzysztof Jakubiak**, Editor-in-Chief, mZdrowie.pl

ROUND-TABLE: International initiatives for public health in the context of post-pandemic conclusions (best practices / experience exchange)

Moderators: **Małgorzata Bogusz**, President of IRSS Foundation, European Economic and Social Committee Member and **Bartosz Kwiatek**, Polsat News journalist

Keynote speech: **dr Tomasz Latos**, Chairman of the Polish Parliamentary (Sejm) Health Committee

Panelists:

1. **ppłk Łukasz Krzowski**, Epidemiological Response Center of the Armed Forces of the Republic of Poland
2. **Krzysztof Kopeć**, President of Polish Association of Pharmaceutical Industry
3. **Adrian Goretzki**, President of Healthcare Education Institute
4. **prof. Tomasz Hryniewiecki**, Director of National Institute of Cardiology
5. **prof. Karina Jahnz – Różyk**, Head of Department of Internal Diseases, Pneumology, Allergology and Clinical Immunology, Military Institute of Medicine, National Consultant in the field of allergology
6. **Irena Rej**, President of Management Board of „Farmacja Polska” Chamber of Commerce
7. **Maarten Van Baelen**, Executive Director of the Plasma Protein Therapeutics Association (PPTA)
8. **adw. Grzegorz Mączyński**, advocate, pharmaceutical law and healthcare expert

PANEL IV: Civilization diseases – scale, determinants and threats vs. treatment opportunities

Maciej Miłkowski

- Currently, we face several types of epidemics:
 - COVID-19 pandemic;
 - an „epidemic” of chronic (non-communicable) diseases that have become diseases of civilization: cardiovascular disease, diabetes, obesity, chronic renal failure, cancer, ophthalmic diseases, and rheumatological diseases;
 - an „epidemic” related to the failure of some members of society to accept basic scientific knowledge about vaccination, among others, and to question basic medical issues.
- Recently, there has been a regress in the diagnosis and effective treatment of patients with cardiovascular diseases, such as hypertension and heart disease (ischemic heart disease, myocardial infarction). We have seen good results in the care of cardiac patients over the past 20 years, but this has changed, including due to the negative impact of the COVID-19 pandemic – patients, fearing infection in the hospital or the primary healthcare facilities (POZ), did not show up for tests and follow-up visits, including for heart attack treatment.
- Today, more and more patients are suffering from hypertension. Cardiovascular diseases continue to be the reason for shorter lives (mortality and excess mortality), especially among men. In 2022, we will want to act following international guidelines to effectively treat cardiac patients. We have a huge backlog of pharmaceutical companies that are not able to register drugs according to the guidelines of scientific societies – I hope that in this regard we will be able to do a lot, as the law enables us to do so – and when it comes to these basic elements – heart disease, heart failure – they will achieve great success. Currently, together with the Institute of Cardiology, we are starting the Polish Cardiac Network pilot project.
- We face a problem of underestimating the number of patients with diabetes – it is assumed that about 20-25% of people do not know that they have diabetes. There is low awareness in the population of the prevention and symptoms of this disease.
- It can be stated that now there is an obesity epidemic – more than 90% of men and 70% of women in Poland are overweight or obese.
- We also face the problem of late diagnosis and low public awareness of chronic kidney failure – a lot of people start treatment with kidney dialysis as the disease stage is already advanced.
- Oncology and the extremely expensive treatment of patients associated with it is also a big problem.
- Taking care of the good condition of the organs of sight and motion is extremely crucial in maintaining interpersonal relationships.
- People know that something is unhealthy for them, yet they very often risk their health and even their lives. They don't accept the recommended treatment, for instance, in hypertension, and diabetes – patients consciously do not accept and do not follow medical recommendations.
- Problems of paramount importance are alcohol abuse and smoking – people consciously act against their health, knowing that most oncological diseases are related to these stimulants.
- Improving health depends on changing lifestyles – it is extremely vital to increase physical activity, maintain a proper diet, and beware of those stimulants.

- Shortly, we will have a problem with financing the treatment of civilization diseases – they will also pose a major challenge to the country's development and further economic growth. However, living a healthy and fit life is essential for proper and satisfying functioning in society or in the labor market.

Successes, challenges, and proposed solutions in cardiology

prof. Robert Gil

- Polish cardiology is used to achieve success. First of all, we can boast of a network of centers fighting heart attacks, although given new technologies, including modern drugs, these centers lag a bit behind the needs of patients. We also have successes in establishing Coordinated Care Programs, enabling us to approach and treat patients holistically. For example, a program dedicated to patients, who have suffered a myocardial infarction is proving to be very successful, extending life by as much as three years in several dozen to several dozen percent. These are very good models worth following. More groups of patients could benefit from this.
- It would also be appropriate to return to the Coordinated Care Program for Patients with Heart Failure, which was approved in 2018, with the indicated budget. A special group of patients requiring such a coordinated approach is composed of patients with atrial fibrillation. In their case, the prevention of strokes plays a huge role.
- I hope that the National Cardiac Network will also bring positive results for patients. However, this is a program that may increase the number of procedures in certain areas of cardiology, nevertheless, it will not contribute to increasing the self-awareness of our society, which, like health-promoting education, is virtually non-existent in Poland today.
- It's high time to bring together the needs of all generations in cardiology. We currently treat pediatric cardiology more as a part of pediatrics and deal mainly with adult cardiology, completely forgetting about the cardiology of the elderly. First, we should start raising awareness of the prevalence of certain disease entities in our population by educating the youngest. Second, we should diagnose the real health needs in cardiology and determine where we can act preventively and where we can intervene. Cardiology is one of those fields of medicine, right next to diabetology, where we can achieve spectacular results with preventive measures.
- Atherosclerotic cardiovascular diseases have for years remained a diagnosed „pandemic“ of today's civilization. And while we have various achievements, for example, in the area of hypercholesterolemia, such as drug programs for patients with congenital hypercholesterolemia and after myocardial infarction with hypercholesterolemia, they remain ineffective, due to insufficient public response. The criteria for drug programs should be loosened, increasing their access to patients. There are, of course, costs involved, but only the introduction of a comprehensive approach to the patient at an early stage of the disease offers a chance to stop this modern „pandemic“ in cardiology.
- Once a patient has been diagnosed, it is necessary to manage the patient effectively, to diagnose the so-called early atherosclerotic patients – after a heart attack or stroke, as well as to look at their families to pre-empt further such cardiovascular episodes. These are the tasks facing our country's healthcare system. What we have now is the so-called firefighting, i.e pouring money from one area to another, just to deal with the problem on an ad hoc basis. We need a long-term perspective and strategy and the implementation of a preventive and educational approach in society from an early age.

The role of education and prevention in civilization diseases of the lungs, heart, and diabetes

prof. Paweł Śliwiński

- Lung diseases can be divided according to various criteria – including distinguishing infectious diseases and chronic non-communicable diseases. The magnitude to which respiratory infections can grow has been particularly vividly demonstrated by the pandemic, being the main cause of hospitalization, intubation, and most deaths in COVID-19.
- A disease on the borderline of pulmonology and oncology is lung cancer, which is the leading cause of death from oncological diseases in both men and women. Poland's statistics compared to other European countries are abysmal in this regard – we have one of the lowest 5-year survival rates among lung cancer patients, as well as one of the highest incidence rates of the disease in society. Awareness needs to be raised among both physicians and the general population about the functioning of the Lung Cancer Early Detection Program, which has effective treatments for lung cancer, including molecularly targeted therapy or immunotherapy, starting in 2020. Few people are aware of this, which is why the program is underperforming. Invariably, prevention also plays a key role, as early detection of lung cancer, provides the greatest opportunity for effective treatment.

dr Tomasz Latos

- For years we have been talking about the problems of civilization diseases. Changes in this issue are occurring at an extremely slow pace – it is required to make a breakthrough in this area. In addition to focusing on treating diseases that have already occurred, we should also be concerned with prevention and health promotion. In the case of heart disease and diabetes, there should be a much more active promotion of physical activity and healthy eating habits from an early age. The toll of smoking traditional cigarettes also needs to be increased boldly. Doing so will make some people stop smoking, which in the long run will translate into lower rates of COPD and lung cancer, among others.

Challenges and solution concepts in diabetology

prof. Leszek Czupryniak

- In April 2021, the Chancellery of the Prime Minister of Poland unveiled data showing that half of our country's citizens gained 6 kg over the course of 2020. Getting rid of this excess weight will be very difficult – what's more, it is already resulting in an increased incidence of diabetes, cardiovascular diseases, and hypertension – a number of civilization diseases that we do not know how to cure. Urgent action must be taken to increase the medical staff, improve the infrastructure where patients are treated, and improve access to effective therapies and modern medical equipment.
- Recently, a range of good things have happened in diabetology in terms of reimbursing therapies for patients with diabetes – there is, however, still more that can be done. For instance, instead of reimbursing very low-cost drugs, it would be appropriate to address reimbursement of more expensive, innovative drugs, which are emerging in abundance in every field of medicine.
- Entirely new concepts are required at every level of healthcare. First and foremost, new solutions should be developed for health education, which is currently lacking but is crucial for chronic civilization diseases.

- The Ministry of Health has prepared a draft of pharmaceutical care for patients at the end of 2020. From the point of view of diabetologists and doctors dealing with chronic diseases, this is a very good solution and requires close cooperation between specialists and pharmacists. It would relieve the burden on the healthcare system, but, above all, it would greatly benefit patients.

Challenges and role of preventive healthcare in pediatrics

prof. Teresa Jackowska

- Health prevention should start with pregnant women. Then, health-promoting teaching should be introduced already among preschool and school-aged children – without this, we will soon have a great epidemic of civilization diseases. An extremely important and necessary solution is the planned introduction of dietary advice for children within the framework of primary healthcare, as well as efforts to increase physical activity among children and adolescents. Here, close cooperation between doctors and teachers is essential, so that children are not exempted from P.E. classes in schools, but rather these lessons are adapted to people with different levels of physical fitness.
- A major problem in pediatrics is the staff shortage in hospitals (the rules of financing this specialization would have to be changed), as well as the overuse of giving antibiotics to children by primary care physicians. Currently, most infections are viral diseases, so if we do not stop treating them with antibiotics, we will soon have, among others, a rash of respiratory diseases.
- A child's health balance is not being carried out properly, yet it is a key moment when we can find out the child's various organic and mental problems – particularly, taking into account that more and more children and adolescents nowadays are struggling with mental problems – a British study showed that the number of young people suffering from depression and other types of mental disorders has recently increased to double.
- The Immunization Program also needs to be changed. At least in one year – in one adolescent age group, i.e. 11-12 or 12-13 years – free HPV vaccination should be introduced, with the condition of vaccination before the age of 15. It is true that as of November 1, 2021, an HPV vaccine with a 50 percent reimbursement appeared on the reimbursement list, but the vaccine must be reimbursed 100 percent to make vaccination against the virus more widespread.

Challenges in inflammatory rheumatic diseases – how to effectively get back the patient to activity?

prof. Brygida Kwiatkowska

- Inflammatory rheumatic diseases can occur at any time in life, from childhood to adulthood. We completely wrongly associate them with the degenerative disease in seniors.
- At the moment we have two complicated situations – first, life expectancy is increasing, as a result of which we are seeing a second peak in the incidence of rheumatoid arthritis (RA) in late life. Second, SARS-CoV-2 infection can induce the development of inflammatory rheumatic diseases – here again, RA is the most common of them. Moreover, the pool of people with inflammatory rheumatic diseases will go up due to the co-occurrence of the inflammatory agent and obesity. This, in turn, exacerbates the risk of cardiovascular complications and the onset of diabetes, oncology, and lung disease. Until we don't accelerate the detection and treatment of patients with inflammatory rheumatic

diseases without the chronic use of glucocorticosteroids, which currently have very limited use in rheumatology, the number of patients requiring multispecialty care will continue to grow, generating higher therapy costs.

- We are pleased that Minister Miłkowski supports the inclusion of a pilot Program for the Care of Patients with Early Arthritis, thereby increasing the ability to diagnose patients. Recently, in cooperation with the Ministry of Health, we have had the opportunity to introduce several modern molecules that can reduce the inflammatory process very effectively, and thus not cause comorbidities or delay their onset. The more therapeutic options we have, the better young patients will function in society.

Civilization diseases as a driver for medical development – the role of coordinated healthcare

prof. Jarosław Fedorowski

- Civilization diseases are an opportunity that has already caused incredible development in medicine and healthcare. Thanks to technological advances, we are living longer and longer, keeping the elderly in good health condition.
- The advances resulting from the introduction of coordinated healthcare are of great value to the patient, who is diagnosed in detail and then treated in a multidisciplinary, staged, long-term manner, either on an outpatient or inpatient basis, as required. An example of effective coordination in healthcare is the very successful KOS-Zawąt (Myocardial Infarction) Program. Hence, we should keep on going and apply healthcare coordination mechanisms on a large scale.

Actions prioritization in civilization diseases

dr Małgorzata Gałązka-Sobotka

- Unfortunately, any healthcare system, when facing challenges, must be aware of the limitations arising from the limited availability of human, organizational, and financial resources. We live with these limitations every day, and we know that even if we took very intensified measures to reduce them, they would still be with us. So it is necessary to prioritize those civilization diseases that we should address first. Then we should go to the data. The diseases of civilization are today the biggest burden from the point of view of both the healthcare system and social and economic capital, which should be taken into account when prioritizing the sectors.
- In the healthcare system currently we are primarily focused on restorative medicine. Of course, when the disease occurs, medical intervention is very important from the patient's perspective, as well as that of the economy and society as a whole. However, we should increase the role and importance of preventive healthcare. We need to focus on education throughout the life cycle. In addition to raising awareness among children and adolescents, we must also educate parents and seniors, because their attitudes and behaviors in life determine whether we can effectively reduce the risk of many diseases, i.e., apply phase 3 prevention skillfully. We have a good diagnosis of the problems, but we also need consistency in implementing effective, proven solutions, which at this point, unfortunately, we lack.
- An important issue is that the obesity problem does not find its place in epidemiological statistics. The reason is that obesity is underestimated and not diagnosed, even though it is the cause of many other civilization diseases, such as diabetes, heart disease, cancer,

and kidney disease. One can also talk about 200 obesity complications, which should mobilize all of us to take decisive action to address this disease entity seriously defining a diagnostic and therapeutic standard for all age groups.

The role of AOTMiT in the prevention and control of civilization diseases

dr n. med. Roman Topór-Mądry

- Agency for Health Technology Assessment and Tariff System acts as a humble advisor to the Minister of Health and is currently analyzing a range of issues, including access to treatment for RA, HPV, and diabetes. The Agency is also working on several coordinated care programs and even a complete remodeling of treatment to achieve maximum efficiency. We are also preparing recommendations for health policy programs for local governments, including on the problem of obesity, its prevention, and risk factors, while in 2022 we plan to prepare a valuation of pediatrics financing. I am glad that we are all looking at health holistically, seeking the best possible solutions.

PANEL V: European solidarity and the diversity of its dimensions – rare diseases and their treatment as an element of the civilizational and ethical foundation of modern Europe.

Specifics of rare diseases and important European initiatives taken for their benefit

prof. Krystyna Chrzanowska

- Rare diseases are chronic, complex, and progressive, causing disability, reducing the quality of life, and often shortening life expectancy.
- The low prevalence of rare diseases means that the experience of those who encounter them is often too limited to be called an expert experience. Access to research-based knowledge is fragmented and limited, and patient care is often inadequate. In the case of rare diseases, the European Commission's recommendations to undertake effective multi-center collaboration at the international level are having a tangible effect.
- Activities within the European Union for rare diseases have been going on for more than 20 years. The first initiative taken in this area was the creation of the Orphanet platform in 1997. In contrast, rare diseases gained their special status under EU legislation in 1999, when the European Commission adopted the European Joint Programme of Action on Rare Diseases (EJP RD), as part of its public health activities. A December 1999 regulation of the European Parliament and the European Council noted that patients suffering from rare conditions should be entitled to the same quality of treatment as other patients. Another milestone in the area of rare diseases was the adoption of the European Partnership on Rare Diseases under Horizon Europe Partnerships, the European Partnership Program. Its idea is for the European Union to work together with private and public partners to support the development and implementation of research and innovation programs. A team has been in place since April 2021, working to develop a new program for rare diseases for the next few years.
- We are very pleased that the National Plan for Rare Diseases has been created. Its goal is to improve the diagnosis of rare diseases and to develop comprehensive patient care – via the establishment of expert centers, selected from among the institutions belonging to the European Reference Network, whose task will also be to enter data on diseases

into the Rare Disease Registry. In the first half of 2022, we should work out with the Ministry of Health, the Agency for Health Technology Assessment and Tariff System (AOTMiT), and the National Health Fund for the financing of large-scale tests, which greatly accelerate the diagnosis of rare diseases. We hope to get this done as soon as possible, as the continued success of the Rare Disease Plan depends on it. Unfortunately, I also see potential problems that we may encounter in its implementation, i.e., a large shortage of geneticists whose task will be to provide comprehensive patient care at expert centers.

The concept of European solidarity and its role in the area of rare diseases

dr Tymoteusz Zych

- European solidarity is a value that cements our identity as Europeans, and it means bending over for the vulnerable, disadvantaged, minority, which in this context is represented by those struggling with rare diseases. In Poland, solidarity has its special dimension and is based on the conviction that there is a community that is the sum of its parts, each of which requires support, even if it will sometimes be unprofitable or associated with immediate inconvenience. When we talk about rare disease therapy, we are not only talking about solidarity but also about anti-discriminatory action. This is a value that will be of great importance from the EU perspective.
- Over the past few years, there have been significant changes in the field of rare disease therapy in Poland, including the issuance of a number of positive reimbursement decisions, the adoption of the Plan for Rare Diseases in August 2021, making this therapeutic area one of the priorities of state health policy, and gathering a whole team of experts around it.
- Rare disease therapy, i.e. the development of innovative solutions and drugs, usually involves similar financial outlays as any other therapy, but due to the relatively small number of patients, the unit price of treatment is much higher. Therefore, from a budgetary perspective, it involves a certain sacrifice, but looking at the situation in which 2-3 million people in Poland are in a difficult health situation and, consequently, in a tough social situation, it is difficult to doubt that this anti-discrimination action has a purpose and sense and that prioritizing rare disease therapy is the right decision.

Health policy and reimbursement process in rare diseases – challenges for 2021-2023

Recently, about 50 of Minister Miłkowski's reimbursement decisions concerned reimbursement access to medical technologies for rare diseases – both for blood cancer and non-oncology conditions.

Maciej Miłkowski

- Rare diseases affect small groups of patients and use more expensive technologies – now it is the time for us in Poland to also focus on the diagnosis and treatment of this group of diseases.
- In rare diseases, we are currently seeing a huge rash of new drug technologies. Very often, the approvals of these drugs are based on fairly low scientific evidence, with the need for further monitoring of the clinical trial to see if the drug is effective when administered to patients who did not participate in the trial. At the same time, pharmaceutical companies do not feel that they can reimburse 100% of the costs incurred by the state as a result of reimbursing the therapy, in case the European Medicines Agency (EMA) does not renew the drug's approval decision in the future.

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- What is relevant in reimbursement processes in rare diseases is that it is ethically questionable to prove the efficacy of a first drug, often minimally better than a placebo, and to fund 1-2 million of the annual cost of therapy even for a group of 20-30 people. This is because it is an inefficient use of funds that could be allocated to therapies whose efficacy is not in doubt. In rare diseases, several therapies with high clinical efficacy are available, and in such cases, the Ministry of Health makes every effort to obtain good terms for their funding. In rare diseases, we are often unable to predict the exact need of patients for a particular drug, so we buy it for all people in Poland diagnosed with a particular rare disease. We then sign risk-sharing agreements, but we try to cover by reimbursement all disease entities in the rare disease group for which we see the possibility of effective therapy.
- The treatment options for patients with rare diseases are matched by Poland's best ongoing newborn screening program, which is used, for example, for spinal muscular atrophy (SMA). This program puts us at the forefront of Europe, as Poland is the only country that will implement newborn screening for SMA nationwide starting in 2022.
- The situation is worse for rare diseases in adults. In their context, the Plan for Rare Diseases is applicable, which calls for the strong implementation of effective diagnostics and the creation of centers of expertise. In some conditions, such centers of excellence are located in the United States. In such cases, it doesn't make sense to treat patients in Poland; we must know where they can be treated, ensuring that they have access to therapy. I also hope that we will soon begin to effectively implement the rare disease solutions included in the Medical Fund.

Cooperation of the Parliamentary Group for Rare Diseases with the Ministry of Health

Barbara Dziuk

- Any achievements in the area of rare diseases would not be possible without the involvement of scientific experts and parents of children struggling with such conditions. Unfortunately, patients with rare diseases belong to a socially excluded group, even though there are several million people in Poland.
- Our gratitude for the progress in the diagnosis and treatment of rare diseases is also to Minister Miłkowski and the Ministry of Health, who conduct an effective dialogue, approach this group of patients with great empathy, and want good changes for them. The Parliamentary Group for Rare Diseases expresses further readiness to act and develop joint positions with the Ministry of Health. The role of the Group remains to propose ready-made solutions and set the course of action to improve the comfort of patients suffering from rare diseases.

The perspective of the Commissioner for Patients' Rights on rare diseases in Poland

Grzegorz Błażewicz

- Rare diseases are a huge challenge for the healthcare system not only in Poland but also throughout Europe. Most of the conditions in this group are incurable or very difficult to treat. In Poland, the discussion on developing an appropriate system of care for patients with rare diseases has lasted for many years, involving all groups of stakeholders – from patients to clinicians, policymakers, and health system experts.
- The Commissioner for Patients' Rights (RPP) carefully listens to the numerous voices of patients alarming about the lack of effective system support in the area of rare diseases. Patients pointed out, among others, the problems of reaching the center where the therapy is provided, the inability to administer the drug at a healthcare facility closer to their place of residence, limitations due to the available drugs within a given drug program, as well as the long diagnostic path. As it turned out, primary care physicians (POZ) have limited knowledge of rare diseases, resulting in significant delays in diagnosing a patient. What's more, patients have often had to circulate through multiple specialized centers before hearing the right diagnosis.
- Over the past few years, we have seen the emergence of new therapies in Poland as an important tool in the fight against rare diseases. Some of them have been publicly funded, while at the same time, some patients have received individual approval to cover the cost of treatment. However, there has always been and still is a group of patients who can't count on even partial funding for these extremely expensive therapies. For this reason, we, as RPP, have always been sincerely interested in the implementation of the National Plan for Rare Diseases. When the Ministry of Health published its draft in March 2021, the RP also provided comments on its content, as part of the public consultations taking place. These comments were clarified in cooperation between the RPP and NGOs.
- Cooperation with patient organizations has always been extremely valuable to us, which is why in early 2020 the RPP established the Council of Patient Organizations, which now includes 106 patient organizations. An example of such cooperation was our joint efforts to introduce a newborn screening program for SMA. It was at the request of NGOs that we applied to the Minister of Health, the national consultant, and the National Health Fund on this issue. The program has been implemented, which we perceive as a great success for all circles striving for its introduction.
- Also contributing to significant achievements in the field of rare diseases was the introduction, after many years of waiting, of the National Plan for Rare Diseases, the appointment by the Minister of Health of a new national consultant in clinical genetics – Prof. Anna Latos-Bieleńska – and the successes of the diagnostic and scientific activities of the Department of Medical Genetics at the Children's Health Center in Warsaw, managed by Prof. Krystyna Chrzanowska. All this means that currently, in terms of diagnosis and treatment of rare diseases, Poland – compared to other European countries – is performing not too badly.
- Despite the successive improvements in the situation of patients with rare diseases, the Office of the Commissioner for Patients' Rights continues to receive indications of how much remains to be done in this area. However, Grzegorz Błażewicz expressed hope, supported by numerous indications, that further improvements to the system of care for rare diseases will soon be implemented.

Successes and challenges in rare diseases from the patients' perspective

In April 2021, the National Orphan Forum conducted an audit of patient needs. According to it, 85 percent of patient organizations demonstrated a need for access to rare disease diagnostics, particularly genetic diagnostics. 76 percent stressed the need for changes in the system of care for patients with rare diseases, and 66 percent indicated the need to improve access to reimbursement for medical technologies – both drugs and non-drug technologies.

Stanisław Maćkowiak

- We are moderately optimistic when looking at what has happened so far in the area of rare diseases. It should be noted that we have been creating the Plan for Rare Diseases since 2008. Of course, it is a huge success that it has now been adopted and started to be implemented. In addition, in the Health Priorities Ordinance, the scope of diagnosis and treatment for rare diseases was indicated as one of the priorities. We hope that the other ideas that have been set out in the Plan for Rare Disease technical document will also be implemented. There is a lot of determination on the part of the Ministry of Health to do so, and Minister Miłkowski's assertion that the time for reimbursement in rare diseases has arrived is also very promising. We have already seen a marked increase in positive reimbursement decisions for therapies in rare diseases over the past 3 years. We do hope that this trend will not only be maintained but also strengthened and significantly accelerated. We must remember that currently, Polish patients have access to about 25% of drugs for rare diseases. Medicine development is progressing very rapidly, and as a result, more and more new technologies will arrive. The availability of therapies for rare diseases in Poland must increase at an even pace so that we don't lag behind other European countries in this regard, where access to innovative medical technologies is currently much greater.
- The scope of the needs of rare disease patients is overwhelming. It is partially enshrined in the Plan for Rare Disease, but only addresses medical issues. The needs of this group of patients, however, go beyond drug treatment, which is only offered to about 5 percent of patients. The remaining patients are provided with treatment and care options in other formats. Both social and educational needs are excluded from the Plan for Rare Disease. We have left these elements for future years, as it is not possible to address all needs of rare diseases at the same time. In the next 2 years, we plan to check what has already been achieved and to what extent the Plan has been implemented, and then we will create specific tasks for it regarding rehabilitation, social welfare, and educational issues.
- It is worth noting that before the creation of the National Plan for Rare Diseases, patients struggling with rare diseases were also taken care of. However, the Plan introduced system solutions, providing patients with comprehensive care in its full scope, so that they will have a chance to improve their situation and lead the same life as other citizens of our country.

Access to diagnosis and treatment of rare blood diseases

dr hab. Anna Szmigielska-Kapton

- A large group of hematological rare diseases consists of acquired diseases, where the genetic diagnosis will not be entirely applicable. In rare blood diseases, it is necessary to improve good diagnosis and modern, often targeted treatment, as well as a sense of security and stability for the patient during treatment. It is critical to ensure the continuation

of therapy if it is well tolerated and effective. This is particularly important in the case of life-saving treatment, in which case uninterrupted access to therapy is absolutely crucial.

- For the ultra-rare acquired disease, as, for example, paroxysmal nocturnal hemoglobinuria (PNH) the drug program ended rather abruptly in April 2021. As a result, the treatment continues in patients who are undergoing therapy, while we can't include it in new patients. We have a big problem with how to proceed with this group of patients when we know that there is an effective life-saving treatment available, but at the same time, it is not reimbursed. Some PNH patients end up in clinical trials, so they are treated outside the National Health Fund. I am very pleased that the Plan for Rare Diseases has been created, which will result in a comprehensive approach to the problem of this group of conditions. At the same time, I hope that treatment continuity for PNH patients will be ensured, and that access will also be granted to new patients.

Challenges in the treatment of hemophilia and other hemorrhagic diatheses – an example of system dualism in rare diseases

red. Krzysztof Jakubiak

- A report published by the Modern Healthcare Institute, „Therapy Development in the Treatment of Hemorrhagic Diathesis,” on the treatment of hemophilia in Poland has exposed various systemic problems in the healthcare field. Currently, there is a kind of system dualism in the treatment of hemophilia in our country. On the one hand, there is a well-functioning drug program for patients under the age of 18, thanks to which minors have access to modern therapies that have been developed and patented in recent years. On the other hand, we have the National Treatment Program for Hemophilia and Related Hemorrhagic Diathesis, aimed at adults, where access to modern medical technology is much more difficult. In Poland, more than 90 percent of the drugs used in the treatment of adult patients with hemophilia are the oldest drugs – plasma-derived, while all other countries in Europe are already moving away from this group of therapeutics in favor of much more modern recombinant and extended-release recombinant therapies. This means that a person who turns 18 is automatically placed under the care of the National Treatment Program, which significantly worsens their access to treatment. Unfortunately, this is a fairly common problem in the treatment of adolescent patients in Poland.
- The process of state-of-the-art therapies entering clinical practice for the treatment of hemophilia in Poland is unfortunately not optimistic. In the last 20 years, the European Medicines Agency (EMA) has registered 22 new preparations for the treatment of this rare blood disease. This looks very impressive, but only from a registration perspective, as reimbursement access is still not the best. There are several reasons for this, first – the purchasing system in the National Treatment Program relies on central purchasing, where there is one winner and price plays a huge role in determining it. Older, less technologically advanced medicines are cheaper, hence they win tenders more easily. The second problem of the healthcare system is the lack of a model for modifying solutions that have already been implemented. This could also be a warning for the fate of the National Plan for Rare Diseases, that one can't be happy that the program has been created, it needs to be constantly monitored and adapted to the changing reality.
- Individualization and flexibility of approach to the patient are also very important, especially within small groups of patients with rare diseases. It is worth noting that this usually does not involve higher costs, since the patients are undergoing treatment anyway. Then we are faced with the choice of whether they will be treated optimally or sub-optimally, whether we will give them the drug in a way that is best for them or one with a lower

efficacy. It is crucial that we do not create excessive bureaucracy, that the framework describing the treatment principles is as broad as possible, and that it is the doctor who can make therapeutic decisions based on his/her experience and clinical judgment. Unfortunately, we are currently not yet able to tailor therapy to the needs of a particular patient who goes beyond the rigid provisions of the program.

- In addition, we have a system problem with small groups of patients in Poland – there are currently no registries for patients with rare diseases. I hope this will change soon.

ROUND-TABLE: International initiatives for public health in the context of post-pandemic conclusions (best practices/experience exchange).

prof. Robert Gil

- Improved infrastructure is required to enable hospitals to simultaneously manage patients „infected” with COVID, including appropriate organizational solutions.
- Adequate informational and educational media campaign
- The debt we have at the moment is related to the fact that patients are extremely afraid of entering a hospital with COVID patients. Patients have locked themselves in their homes and stopped getting examined.
- Besides increasing funds to grow the number of procedures, an education and information campaign is important to better pay healthcare workers.

Małgorzata Bogusz

- Patients affected by the pandemic are mostly oncology and cardiovascular patients.
- A new initiative at the European Parliament level, supported by the European Commission, is the merger of two committees for cardiology and oncology. It is expected to result in a common scenario to take care of patients from the aforementioned groups.

The main challenges of the country's strategic security in the context of pandemics. What should be the priorities to be prepared for future epidemics?

Lt. Col. Łukasz Krzowski

- The Military Health Service is carrying out activities based on lessons learned from the pandemic experience so far, including the expansion of hospital infrastructure, the diagnostic base has been expanded, mobile laboratories, expansion of personnel capacity, the concept of carrying out vaccination of soldiers, preparation of a rehabilitation treatment program for soldiers after COVID.

Maarten Van Baelen

- During the pandemic, we had a 20% reliance on plasma from the United States, a problem that already existed before the pandemic. This has created many challenges for therapies using plasma. There is a need to increase the availability of plasma and its safety.
- There are several countries in Europe where plasma is collected by both the public and private sectors, and this is a good model and pattern for other countries. The European Union is revising a directive on plasma collection and donor safety. This could be an opportunity to pass recommendations on plasma collection in individual countries.
- It would be worthwhile to make some changes and prepare the concept of building appropriate plasma fractionation plants.

Adrian Goretzki

- The government has acknowledged that patient safety depends on the availability of plasma products. In many countries, the continuity of access to medicines was interrupted as a result of the pandemic.
- Donors, mainly people from the United States, reduced the frequency of plasma donations – with the fact that Europe is dependent on US supplies there was a shortage of this supply.
- To avoid such situations in the future it is necessary to collect more plasma. This is the first step, as Poland still has too little plasma collected compared to other EU countries.
- The demand for plasma products is growing as they are used for more and more indications.
- Plasma supply can also be increased by building plasmapheresis centers (both public and private) – as shown by the example of self-sufficient countries. In Poland, awareness of plasma is low.

prof. Karina Jahnz-Różyk

- For several years, home immunoglobulin therapy has been introduced in Poland and appropriate devices are used to administer the drug.
- The pandemic has demonstrated that this home treatment is of particular importance.

Grzegorz Rychwalski

- It is crucial to support the industry and rebuild the pharmaceutical industry, in the context of the availability of active ingredients in Europe, to make medicines affordable every day.
- We hope that funds for the National Rebuilding of the Pharmaceutical Industry will be mobilized to guarantee patient safety.
- The Health Ministry's plan includes references to generic drugs, biologics, active substances, and support for the industry working in this area.

Irena Rej

- Pharmaceutical companies are not invited to join prevention with state initiatives. The Polish industry has been good, albeit generic, but modern, and this support is required.
- Without assistance, pharmaceutical companies acting on their own will cut short innovative programs due to a lack of funds.
- Medical Fund has 6 billion, of which only 25% has been spent. This money should be spent on patients with rare diseases, under the emergency drug bill.
- The legislative mistake that was made a year ago should be corrected.
- Medicine has products that cure but can also be used for prevention.
- An important issue relates to indirect costs, which are not often taken into account – It is said to treat not with cheap medicine, but cheaply. The amount of money spent on sick leave is greater than not spent on drugs.

Cezary Kępa

- It is important to maintain a National Cardiac Registry, so that the treatment results, possible complications, as well as waiting time for some procedures, are known. The data from this registry will be directly linked to the data of the National Health Fund, substantively fully

capable of serving the Minister of Health to make various decisions such as reimbursement or implementation of procedures in therapeutic pathways.

- Successes related to the pursuit of prevention come over time. Our role as the Institute of Cardiology is to plan it in the big picture.
- The pilot project of the cardiac network is meant to show what it looks like at the moment and to learn from the weakest points what needs to be improved.
- What we already know today is that when a patient comes to the primary healthcare (POZ) or cardiologist, s/he gets a ready-made treatment pathway, which enables us to detect risks earlier.

ad. Grzegorz Mączyński

- There are three core areas where law and technology will come together:
- Securing medical infrastructure and critical human resources, responding flexibly to health needs and adapting solutions to the needs of specific populations, and harnessing research projects even more to the ongoing health problems solutions.
- Legislative solutions are plenty, and it is now essential to use them creatively and review those areas that should be possibly complemented or modified to best address the flexible tackling of new health challenges.
- In Poland, we have solutions in the form of pilot programs that we could apply more widely. It is now worth looking for solutions used in other countries and seeing if they are adequate in the context of Poland and worth applying. It is crucial to combine legislation with technology
- We are at a moment of technological convergence – we have a combination of evolution in biology and medicine with information technology. New opportunities are opening up, and while the law doesn't always keep up with this, we need to try to amend it so that these solutions are as effective as possible.
- Competitions and projects supporting research are interesting solutions, so funding goes towards solving practical problems – new treatment methods, and the use of new technologies.

dr. Tomasz Latos

- Referring to Ms. Irena Rej's statement, it is an uncertain future event in the context of the reimbursement law.
- A wider access to therapy, for 75+ medicines must be provided.
- We need to look for new solutions, mobilizing everyone, including health services, sports events organizers, politicians, local government officials, all services involved in health-care or people who, organize city transportation, and journalists.

Bartosz Kwiatek

- Communication is key and should come first – it is the basis of everything, even the exchange of experience.
- Vigilance and alertness are also required, coordination followed by rapid response and thoughtful action.
- We have excellent people, who can prepare a relevant strategy, respond to these challenges, and write a prescription for the future of Europe and Poland.

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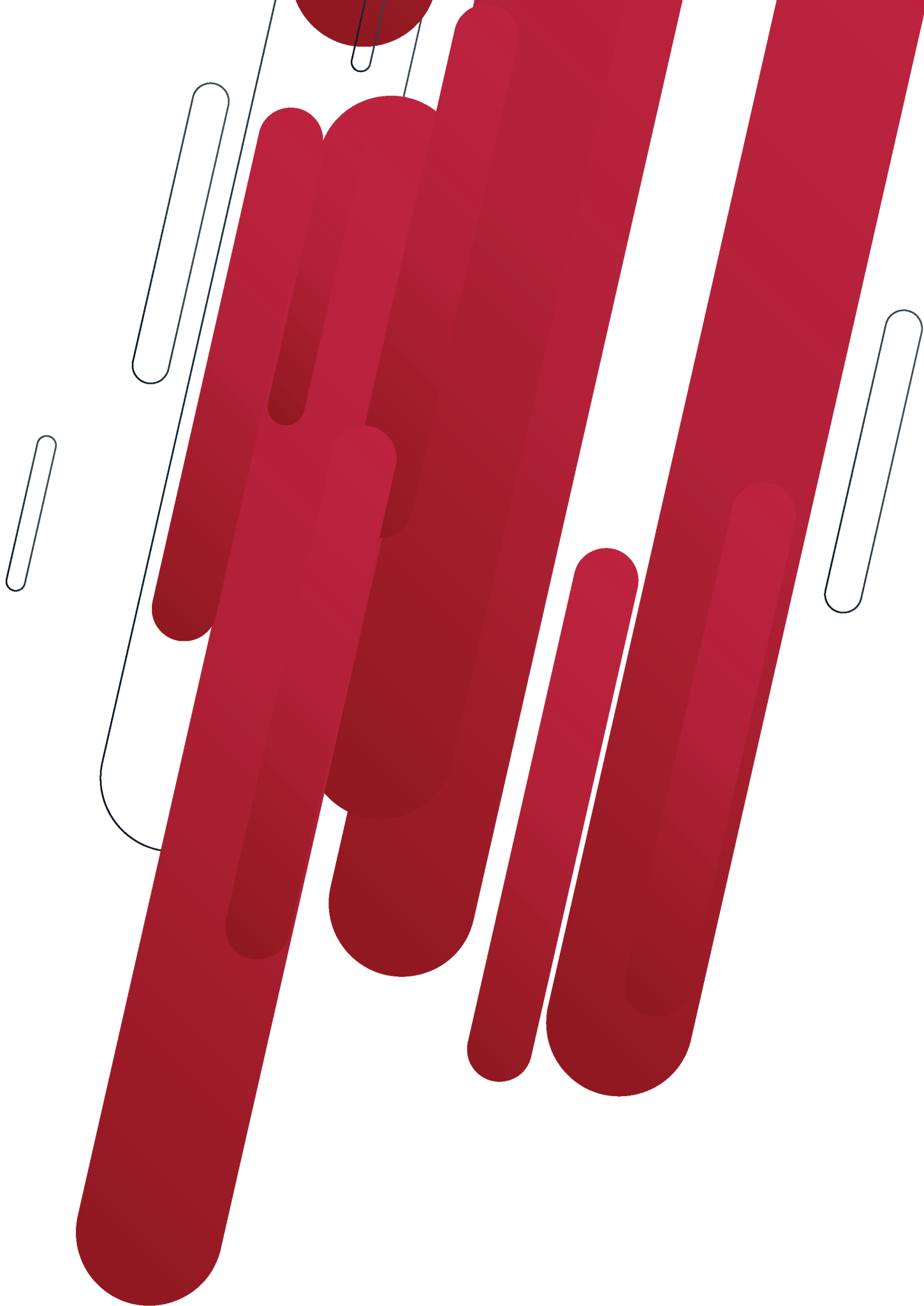
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