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The attitudes of medical students towards rare diseases: A cross-sectional study

Stavovi studenata medicine o retkim bolestima: studija preseka

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Abstract

Background/Aim. Rare diseases are chronic, degenerative and may lead to permanent disability. We aimed to assess knowledge and attitudes of the 3rd and 6th year medical students towards the treatment of rare diseases in Serbia. **Methods.** In this cross-sectional study, two samples of students were questioned for a survey: 350/446 (78.48%) students of the 3rd year, and 242/517 (46.81%) students of the 6th year. **Results.** Sixth year students estimated that they were more informed on the issue analyzed than the 3rd year students (median value of 4 and 3, interquartile range of 3–5, and 1–4, respectively; $p < 0.05$). However, a significant percentage of participants estimated incorrectly the prevalence of rare diseases according to the European Union standards (3rd year – 42.68%, 6th year - 49.55%). Core curriculum subjects were the main source of information on rare diseases (3rd year – 63.14%; 6th year – 92.14%). Our participants agreed that the most important problems are the following: high drug prices, difficult access to drugs and lack of public information. Students found, without any differences, that community access to effective drugs for rare disease should be improved (median value – 10, interquartile range 8–10 in both groups, $p < 0.05$). In order to improve pharmacotherapy of rare diseases in Serbia, the participants suggested establishment of a National Plan for Rare Diseases, approval of more appropriate drugs, simplified access to appropriate medicines, and more rapid diagnostics. **Conclusion.** It is necessary to improve the knowledge and attitudes of medical students towards pharmacotherapy of rare diseases.

Key words:
rare diseases; students, medical; education; serbia.

Apstrakt

Uvod/Cilj. Retke bolesti predstavljaju hronična degenerativna stanja koja mogu dovesti do trajne onesposobljenosti. Cilj ovog rada bio je procena znanja i stavova studenata treće i šeste godine medicine o lečenju retkih bolesti u Srbiji. **Metode.** U studiji preseka, dve grupe studenata ispunile su upitnik napravljen za potrebe ovog istraživanja: 350/446 (78,48%) studenata treće godine i 242/517 (46,81%) studenata šeste godine. **Rezultati.** Studenti šeste godine procenili su stepen svoje informisanosti o retkim bolestima kao bolji u odnosu na studente treće godine (medijana 4 i 3, interkvartilni opseg 3–5 i 1–4; $p < 0,05$). Međutim, značajan procenat učesnika netačno je procenio prevalenciju retkih bolesti definisanu prema standardima Evropske unije (treća godina – 42,68%, šesta – 49,55%). Obavezni predmeti bili su glavni izvor informacija o retkim bolestima (treća godina – 63,14%, šesta – 92,14%). Svi učesnici istraživanja bili su jedinstveni u stavu da su najvažniji problemi obolelih: visoke cene lekova, nedostupnost terapije i nedovoljna informisanost javnosti. Svi studenti bili su saglasni u stavu da društvo treba da poboljša dostupnost terapije za retke bolesti (medijana 10, interkvartilni opseg 8–10, $p < 0,05$). U cilju unapređenja farmakoterapije retkih bolesti u našoj zemlji, učesnici su predložili formiranje nacionalne strategije za retke bolesti, registrovanje većeg broja lekova, pojednostavljivanje procesa nabavke lekova i ubrzavanje dijagnostičkih procedura. **Zaključak.** Neophodno je unaprediti znanje i stavove studenata medicine o farmakoterapiji retkih bolesti.

Ključne reči:
retke bolesti; studenti medicine; obrazovanje; srbija.

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Introduction

It is widely accepted that rare diseases (RDs) affect less than 1 in 2,000, or 5 in 10,000 people [1, 2]. Most of those diseases are due to genetic abnormalities (around 80% of cases). First symptoms appear at birth or in early childhood (around 50% of cases). Diseases are chronic, degenerative and may lead to permanent disability. There are between 6,000 and 8,000 rare diseases and pharmacotherapy is not available for many of them. According to the European Organization for Rare Diseases (EURORDIS), these diseases affect thirty million European Union citizens. It is estimated that there are approximately half a million cases in Serbia [3].

Patients affected with rare diseases, in general, face many problems. Some of these diseases have a devastating impact on all aspects of patients' and carers' lives. Also, healthcare professionals usually lack awareness of the possibility of coming across rare diseases in their professional careers [4]. The consequence is often misdiagnosis and/or delayed diagnosis. It is estimated that it takes approximately seven years to be diagnosed with a rare disease [5]. A patient-oriented coding and classification system ("p-classification") has been recently developed in Germany in order to strengthen patients' efforts and improve exchange of information. However, there is a breakdown in the exchange of information between experts and public on RDs. In addition, the majority of the health workforce is not sufficiently informed on RDs [6].

In Serbia, the problem of the diagnosis and treatment of RDs is currently under public scrutiny. Patients are often misdiagnosed or the diagnosis is delayed due to several problems: lack of awareness among physicians, lack of expertise, unavailability and/or high costs of diagnostic tests. According to the National Organization of Patients with Rare Diseases in Serbia (NORBS), many diagnostic procedures have to be conducted abroad and the process comprises many difficulties: high costs, travel expenses or transportation of biological material [3]. Although national legislation ensures the availability of drugs for those diseases, pharmacotherapy is faced with many problems. Licensed drugs are not always or not readily available and provision of unlicensed medicines is very difficult not just due to legislative obstacles. Also, people affected with rare diseases may require other medicinal devices such as diapers, special nutritional preparations, wheelchairs etc. and these expenses are not reimbursed by the national health insurance. All these obstacles in the treatment and care of patients with rare diseases has led to many charitable actions aimed at collecting donations for the treatment of patients and the involvement of media in raising public awareness of rare diseases. The still no national registry of rare diseases in Serbia, as well as national strategy regarding these diseases [7, 8].

Knowledge about rare diseases among medical doctors is crucial for the improvement of perspectives for those patients. Education on rare diseases contributes not only to more accurate and timely diagnosis and treatment of particular patients, but also improves general aspects of this problem such as planning of national strategy, funding, foundation of expert centres etc. Therefore, the role of undergraduate medical education is essential in providing good foundation for future doctors involved in this issue.

In particular, rare diseases are extensively discussed through various undergraduate courses, but there is not a specific course dedicated to such a problem. We aimed to assess and compare knowledge, awareness and attitudes of the 3rd and 6th year medical students from the Faculty of Medicine, University of Belgrade, towards the issue of rare diseases in Serbia. These two groups of students were selected for particular reasons: 3rd year students complete preclinical, while 6th year students finish clinical curriculum. We tried to establish whether clinical curriculum improved knowledge and attitudes of students regarding this issue.

Methods

Participants

The cross-sectional study was conducted over five days at the Faculty of Medicine, University of Belgrade[*]. Two randomly chosen samples of the 3rd and 6th year undergraduate students were chosen for the survey. The questionnaire was completed by a total of 350 out of 446 (78.48%) students of the 3rd year, and 242 out of 517 (46.81%) students of the 6th year. The participants were recruited during regular classes.

Questionnaire

All the students completed an anonymous questionnaire entitled "Knowledge and attitudes of medical students about rare diseases in Serbia" (Addendum 1). The questionnaire was developed at the Department of Pharmacology, Clinical Pharmacology and Toxicology, Faculty of Medicine, University of Belgrade and reviewed by experts on rare diseases from several clinical fields (neurology, paediatric neurology, gynaecology and internal medicine). It was a pilot questionnaire tested with 50 third year medical students. Pilot test included the same questions as the study questionnaire and revealed appropriate understanding of questions and very low rate of skipped answers. The verbal feedback from the pilot tested students was overall positive. The questionnaire was approved by the Ethics Committee of the Faculty of Medicine in Belgrade (Number 29/XI-14). Both closed and open questions were used when appropriate. The questionnaire consisted of general data (such as gender, age and the average study score), questions about awareness of rare diseases and their treatment, attitudes about problems of these patients, as well as the possibilities of overcoming them. Students' attitudes towards RDs were assessed by the 10-point Likert scale.

Statistics

The results were presented as descriptive statistics (nominal scale) and median value and interquartile range (results scores). A statistical analysis was performed using χ^2-test and Mann-Whitney U test, with a statistical significance level of 0.05.

[*]Most prestigious, biggest and the oldest Faculty in Serbia, on the Shanghai List of Universities.

Results

General characteristics of the participants

General characteristics of the 3rd and 6th year medical students who responded to a questionnaire survey on attitudes towards rare diseases treatment were shown in Table 1. There was a total of 350 3rd and 242 6th year students, with similar gender distribution and similar average grades in both groups.

Samples did not differ in any of the characteristics presented.

Students' self-assessment of rare diseases knowledge (general knowledge and treatment possibilities)

The students were asked to rate their knowledge about rare diseases in general, based on the 10-point Likert scale. As expected, the 6th year students rated their knowledge as significantly better than the 3rd year students (median score 3.0 *vs* 4.0, $p < 0.001$) (Figure 1, Panel A).

The overall quality of healthcare of patients affected with rare diseases in general was rated as low by both groups of students. Based also on the 10-point Likert scale, the 3rd year students' score for this question was 2.2, and the 6th year students' 2.4 ($p < 0.05$) (Figure 1, Panel B).

The complications associated with obtaining drugs for rare diseases in Serbia were graded as significant by both groups of students, but year 6th students opinion was more favorable. Based on 10-point Likert scale, the points ranged from 1 (extremely complicated) to 10 (not complicated at all). The score provided by 3rd year students was 2.2 *vs* 2.7 provided by the 6th year students ($p < 0.001$) (Figure 1, Panel C).

Both groups of students graded lowly the possibilities

Table 1

General characteristics of the study population (3rd and 6th year medical students)

Medical students	3rd year	6th year
Number of respondents	350	242
Sex (male/female), n (%)	109/241 (31/69)	74/168 (30/70)
The year of enrolment to Faculty of Medicine, median values (interquartile ranges 25–75%)	2009 (2008–2009)	2006 (2005–2006)
Average score during the studies, median values*	8–9	8–9

***Score rank 6–10.**

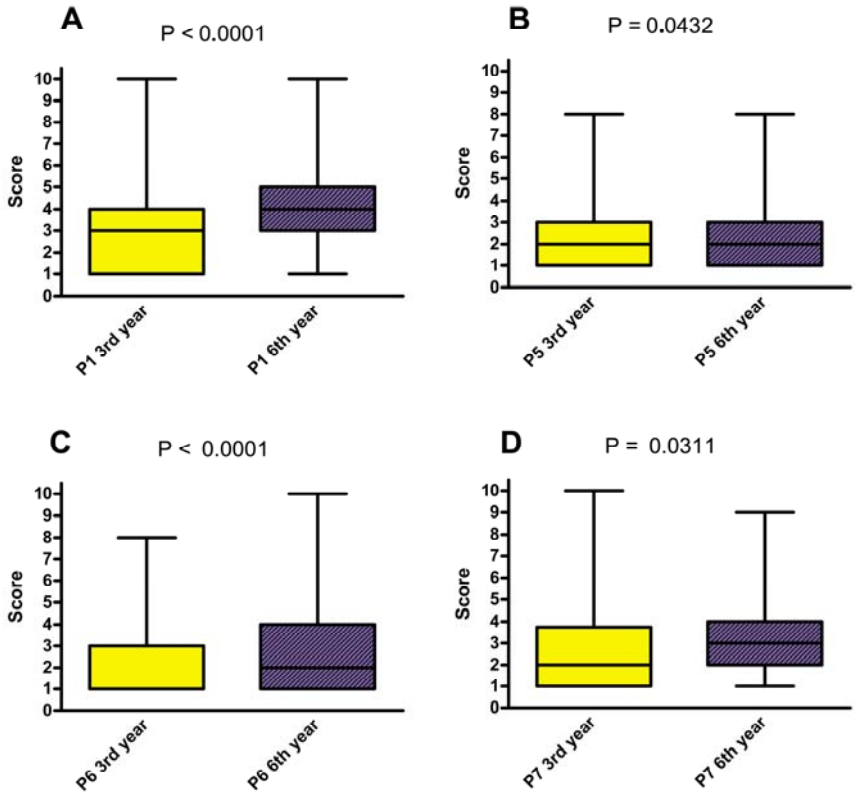

Fig. 1 – The average values of the selected scores (median value, interquartile range 25–75%) of the 3rd and 6th year medical students, concerning their attitudes and awareness towards the issue of treatment of rare diseases in our community. Panel A: Awareness towards the issue of treatment of rare diseases (scale 1–10); Panel B: Attitudes towards the possibilities of obtaining drugs for rare diseases in Serbia (scale 1–10); Panel C: Awareness towards the possibilities of obtaining drugs for rare diseases in Serbia (scale 1–10); Panel D: Attitudes towards the availability of drugs for rare diseases in our country (scale 1-10). The results are shown in a graph box ("Box plot") with a median value and interquartile range (25–-75%). Ordinate: arbitrary score. The significance of the difference between the 3rd and 6th year students was determined by Mann-Whitney U-test.

of obtaining drugs for rare diseases in Serbia. However, the estimate made by the 6th year students (2.8) was significantly more favorable compared to 3rd year students (2.6) ($p < 0.05$) (Figure 1, Panel D).

Objective assessment of students' basic knowledge on rare diseases

The knowledge on the prevalence of rare diseases in Europe was tested with a multiple choice question. The prevalence of rare diseases according to EU standards was correctly estimated by a slightly more than half of participants in both groups of students (3rd year – 57.32%, 6th year – 50.45%, $p > 0.05$).

The participants recognized rare diseases from the list comprising both common and rare diseases, in the following percentages: acromegaly (3rd year – 36.57%, 6th year – 21.07%), haemophilia (3rd year – 35.14%, 6th year – 30.99%), Gaucher disease (3rd year – 48%, 6th year – 83.05%) and Non-Hodgkin's lymphoma (3rd year – 34%, 6th year – 8.2%) (3rd year *vs* 6th year, $p > 0.05$, each). However, the overall distribution of answers differed significantly between the 3rd and the 6th year students, surprisingly in favor of the 3rd year students ($p < 0.0001$).

We also tried to establish which sources of information on rare diseases students mostly used, with the question that allowed more than one answer. The 3rd and 6th students used different sources of information on rare diseases. The former group indicated core curriculum subjects as the main source of information on the rare diseases (for example, Pathology, Pathological Physiology, Immunology, Internal medicine and Neurology) – 63.14%, followed by some other extracurricular sources (television, internet) – 39.42%, and a large number of elective courses – 11.4%, while the latter group cited the same sources in the following percentages: 92.14%, 6.67% and 27.68%, respectively.

Students' attitudes regarding rare diseases as a societal and bioethical issue

The next set of questions aimed to assess students' attitudes regarding rare diseases as a broader societal problem.

Using the 10-point Likert scale, both groups of participants rated the importance of rare diseases in our society as a problem of secondary importance (3rd year 5.9 and 6th year 5.8; $p > 0.05$). Also, using a multiple choice question which allowed more than one answer, they identified particular problems of Serbian patients affected with rare diseases (Table 2).

The students were asked to identify the most competent factors (e.g. medical professional associations, drug manufacturers and distributors, patients associations or state regulatory bodies) which could improve availability of drugs for rare diseases. As the most responsible, both groups of students unanimously identified state regulatory bodies (Table 3)

Regarding measures for improving pharmacotherapy of rare diseases in Serbia, the collaboration between physicians and pharmacists in order to advance the availability of drugs for rare diseases was highly ranked, with no difference between the groups ($p > 0.05$).

When asked to evaluate whether to provide costly medical care for patients with rare diseases or to spend on treatments for more common conditions affecting larger numbers of people, participants mainly supported the former opinion (10-point Likert scale, 3rd year 7.9; 6th year 7.9; $p > 0.05$).

Table 2

The 3rd and 6th year medical students' opinion on the most important problems of patients with rare diseases

The most important problems of patients	Students (%)		
	3rd year	6th year	*p* value
Lack of information among general public	67.71	56.61	< 0.01
Lack of scientific knowledge	31.42	37.19	> 0.05
Lack of access to correct diagnosis	46.28	42.56	> 0.05
Lack of appropriate quality healthcare	49.71	40.08	< 0.05
Lack of registered drugs for rare diseases	41.43	70.24	< 0.0001
Complicated procedures of drug provison	60.00	63. 22	> 0.05
High prices of drugs	86.29	80.58	> 0.05
Other causes	2.00	1.65	> 0.05

Table 3

The 3rd and 6th year medical students' opinion on ways for improving availability of the drugs for rare diseases

Availability of drugs for rare diseases can be improved by greater iinvolvement of ...	3rd year (mean)*	6th year (mean)*	*p* value
The State	8.9	8.8	> 0.05
Drug manufacturers and distributors	7.9	7.8	> 0.05
Associations of patients with rare diseases	6.8	6.7	> 0.05
Medical profession associations	6.0	5.8	> 0.05

***Mean of the 10-point Likert scale (1 – minimal; 10 – maximal).**

However, the majority of participants would refrain from taking responsibility regarding funding treatment for rare diseases, especially at the expense of more common treatments (3rd year - 58.19%, 6th year – 61.99%, $p > 0.05$).

In order to improve the pharmacotherapy of rare diseases in Serbia, the participants suggested the following: raise the general awareness about this topic, well-timed diagnostics, simplified drug approval processes, full implementation of national and international legislation on that matter, tight regulation of drug provision through the private pharmaceutical sector, approval of new drugs for rare diseases, establishment of a National Strategy for Rare Diseases and specific rare disease patient registries (Table 4).

Table 4

The 3rd and 6th year medical students' opinion on ways of improving pharmacotherapy of rare diseases in Serbia

How to improve pharmacotherapy of rare diseases	Students (%)		
	3rd year	6th year	*p* value
Raise general awareness and expertise	30.57	60.00	< 0.0001
Well-timed diagnostics	44.86	63.22	< 0.0001
Simplified procedures for drug provision	50.00	70.66	< 0.0001
Compliance with legislation in its entirety	8.29	17.77	< 0.01
Registration of novel drugs	55.43	69.01	< 0.01
The establishment of the National Plan for Rare Diseases	64.57	74.38	< 0.05
Creating the registry of rare diseases	54.58	53.72	> 0.05

Discussion

This survey shows that the 3rd and 6th year medical students have similar views towards the issue of rare diseases in our community. The latter group was more confident in their knowledge of issues regarding rare diseases, but unexpectedly, the 3rd year students were able to more accurately recognize rare diseases than the 6th year students. This might be due to recent completion of the Pathology and Pathophysiology course by the 3rd year students, in which great attention is paid to some rare diseases. It is slightly disappointing that this knowledge is not retained throughout the complete duration of their medical studies, and not improved during clinical courses. However, the identification of rare diseases is not sufficient to fully evaluate students' knowledge and in this study was used just for rough estimate. The prevalence of rare diseases was correctly estimated by more than 50% of both groups of participant, which indicates the correct understanding of the subject of "rareness". As preferable sources of information, greater percentage of 6th year students chose curriculum subjects and elective courses provided by the Faculty than the 3rd year students. However, the 3rd year students chose television and internet as trustworthy sources of information on rare diseases in significantly greater percentage than the 6th year students. It is possible that the 6th year students are more aware of questionable quality of many internet sources and sometimes sensationalistic media presentations and therefore find university courses more reliable. It is important to note that both groups of students recognized the treatment of patients with rare diseases in our community as a serious problem and expressed significant level of awareness regarding problems of drug provision and low quality of health care.

Although there was a difference in the distribution of responses between the 3rd and 6th year medical students, most of our respondents agreed that the most important problems of these patients were the following: lack of information among the general public, but also among healthcare professionals, complicated procedures for drug provision and the high prices of drugs. Similar observations were noted among healthcare professionals (physicians and clinical pharmacists) in a pilot study performed at the University Hospital "Bežanijska kosa" in Belgrade (unpublished data from our group). A survey entitled "Attitudes of physicians and pharmacists towards the pharmacotherapy of rare diseases in Serbia" was designed and conducted by employees at the Department of Pharmacology, Clinical Pharmacology and Toxicology.

The students find, without any differences, that greater involvement of the state is needed. However, the activities of the state are actually dependent on professionals' (policy makers, members of the regulatory bodies, creators of healthcare strategies) knowledge and attitudes.

The prospective of patients affected with rare diseases in most cases relies heavily on pharmacological treatment, but the lack of interest in pharmaceutical industry in their development is evident [9]. Drugs for many rare diseases have the status of orphan drugs. In the EU, a drug will receive orphan status designation if it is intended for the diagnosis, prevention, or treatment of a life threatening or chronically debilitating condition which affects not more than five people per 100,000 in the Community (Regulation (EC) No 141/2000). Therefore, the development or acquisition of orphan drugs, regarding their costs, is a serious ethical dilemma [10]. Our respondents considered it quite reasonable to invest large amounts of money to treat a small number of patients, but they did not know or refused to answer what was more justifiable for them – to invest in medications for common diseases or to invest in the purchase of drugs for rare diseases. This neutral students' position on prioritizing funds was actually expected and reflects the complexity and

controversial nature of the issue of pharmacotherapy of rare diseases in general [11, 12]. The justification for paying the premium prices of orphan drugs diseases must rest on the question: should we value the health gain to two individuals differently because one individual has a common disorder and the other has a rare disease disorder? The National Institute for Health and Clinical Excellence (NICE) recently recommended that the National Health Service (NHS) should consider paying premium prices for drugs for rare diseases based on three criteria: the severity of the disease, evidence of health gain, and whether the disease is life threatening [13]. But, it should be pointed out that is virtually impossible to assess cost-effectivness of treatments for rare diseases using conventional criteria. Current curriculum provides basic knowledge on symptoms, diagnostic procedures and possible treatment options of rare diseases. However, it would be useful to offer deeper insight into the ethical dilemmas regarding expensive treatment of low prevalence diseases. Medical students should be aware that even the most expensive treatment for rare diseases could actually be cost-effective if it reduces life-time standard health-care costs and improves patients' ability to work or live without assistance.

It is evident that students in Serbia showed interest and willingness to respond to a very sensitive issue of rare diseases. There is a high level of awareness among students that rare diseases are a big medical, socio-economic and bioethical concern. However, certain lack of knowledge was noted among participants in this study, as well as in other countries [4, 14, 15]. Therefore, it is crucial to improve education on rare diseases, but especially on bioethical issues both at undergraduate and postgraduate level [16].

As a non-EU member, Serbia has a chance to be an observer monitor of the EUROPLAN II project (2012–2015) which is designed to support the efforts of national authorities to develop public health strategies on rare diseases throughout Europe following common guidelines. The Integrated EU-national strategy focuses on a few essential building blocks such as information, centers of expertise, registries, and access to medicines [17, 18]. Concerning this, we believe that this is the critical moment for the improvement of education on rare diseases among medical students in Serbia.

Conclusion

Rare diseases are an important bioethical concern. Medical students are aware of that and familiar with the most important problems regarding the diagnostics of rare diseases and enormous costs of research of treatment and available treatment prices. Students are also informed about difficulties with orphan drugs registration and acquisition. However, further efforts should be made in educating medical students, as future participants in health-care policy making, on complex matter of cost-effectiveness of orphan diseases treatment.

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Addendum 1

THE KNOWLEDGE AND ATTITUDES OF MEDICAL STUDENTS IN SERBIA CONCERNING RARE DISEASES

THE SURVEY IS ANONYMOUS

Thank you for participating in our survey. We assure you that you will not have any negative consequences for participation and that we will respect your privacy.

The aim of this study is to investigate the knowledge and attitudes of medical students concerning the treatment of rare diseases in our community. The results will be used in the development of scientific research, exclusively. The authors of this survey are pharmacologists employed at The Departmennt of Pharmacology, Clinical Pharmacology and Toxicology, Faculty of Medicine, University of Belgrade.

We ask you to write legibly and to honestly answer all questions that is the only way your answers will have value.

GENERAL CHARACTERISTICS:

SEX (please circle) M F

YEAR OF BIRTH ----------

YEAR WHEN THE STUDY STARTED (enroll) ----------

CURRENT YEAR OF STUDY (enroll) ----------

AVERAGE SCORE DURING THE STUDIES (round off)

< 7 7–8 8–9 9–10

SCORE IN PHARMACOLOGY AND TOXICOLOGY (if you passed this course) ----------

THE ATTITUDES REGARDING THE TREATMENT OF RARE DISEASES IN SERBIA:

HOW DO YOU RATE YOUR CURRENT KNOWLEDGE ABOUT RARE DISEASES IN GENERAL?

(Mark with X)

(poor) (excellent)

HAVE YOU HAD THE OPPORTUNITY TO MEET SOMEONE WHO IS SUFFERING FROM A RARE DISEASE?

1. YES 2. NO 3. I DON'T KNOW

THE PREVALENCE OF RARE DISEASE IN EU COUNTRIES IS SMALLER THAN:

1. 1/100 2. 1/500 3.1/2000 4. Something else ----------

COULD YOU RECOGNIZE RARE DISEASES FROM THE FOLLOWING LIST? (round off)

1. Acromegaly 2. Osteoporosis 3. Hemophilia

4. Multiple Sclerosis 5. Gaucher disease 6. Schizophrenia

6. Familiar breast cancer 7. Non Hodgkin's lymphoma

THE MAIN SOURCE OF INFORMATION ON RARE DISEASES, ACCORDING TO YOU, ARE?... (enroll)

1. core curriculum subjects (specify them) ----------

2. elective courses (specify them) ----------

3. some other, extracurriculum souces (specify them) ----------

HOW DO YOU RATE THE IMPORTANCE OF SUCH AN ISSUE IN OUR COUNTRY? (enroll X)

(I don't consider it important) (very important)

HOW DO YOU RATE THE POSSIBILITIES OF OBTAINING DRUGS FOR RARE DISEASES IN SERBIA? (enroll X)

(very bad) (excellent)

ACCORDING TO YOU, THE MOST IMPORTANT PROBLEMS OF PATIENTS SUFFERING FROM RARE DISEASES ARE?

(you may select more than one answer)

1. Lack of information among the general public

2 Lack of scientific knowledge

3. Lack of access to correct diagnosis

4. Lack of appropriate quality healthcare

5. Lack of a sufficient number of registered drugs

6. Complicated procedures of drug provison

7. High prices of drugs

8. Lack of observance of legislation

9. Unavailability of these drugs in private pharmacies

10. Other ----------

ARE YOU AWARE OF THE POSSIBILITIES OF OBTAINING DRUGS FOR RARE DISEASES IN SERBIA? (enroll X)

(not at all) (fully aware)

THE AVAILABILITY OF DRUGS FOR RARE DISEASES CAN BE IMPROVED BY GREATER INVOLMENT OF...

MEDICAL DOCTORS

(have no impact) (crucial role)

CLINICAL PHARMACISTS

(have no impact) (crucial role)

PHARMACEUTICAL COMPANIES AND PHARMACIES

(have no impact) (crucial role)

THE STATE

(has no impact) (crucial role)

ASSOCIATIONS OF PATIENTS SUFFERING FROM RARE DISEASES

(have no impact) (crucial role)

HOW DO YOU RATE THE IMPORTANCE OF COLLABORATION BETWEEN PHYSICIANS AND PHARMACISTS IN ORDER TO ADVANCE THE AVAILABILITY OF DRUGS FOR RARE DISEASES?

(no impact) (crucial role)

RARE DISEASES ARE DISEASES WHICH, WITHOUT APPROPRIATE THERAPY, MOST OFTEN, LEAD TO A PERMANENT DISABILITY. HOWEVER, THE TREATMENT OF PATIENTS SUFFERING FROM RARE DISEASES IS EXTREMELY EXPENSIVE. DO YOU FEEL THAT IT IS ETHICALLY JUSTIFIED TO SPEND A LOT OF MONEY FOR A SMALL NUMBER OF PATIENTS?

(not at all) (fully justified)

WOULD YOU (IN A POSITION TO MAKE SUCH DECISIONS) PROVIDE COSTLY MEDICAL CARE FOR PATIENTS SUFFERING FROM RARE DISEASES RATHER THAN SPEND MONEY ON TREATMENTS FOR MORE COMMON CONDITIONS AFFECTING A LARGE NUMBER OF PEOPLE?

1. YES 2. NO 3. I DON'T KNOW/NEUTRAL POSITION

ACCORDING TO YOU, HOW CAN PHARMACOTHERAPY OF THESE PATIENTS BE IMPROVED IN OUR COMMUNITY?
(you may select more than one answer)

1. Raise general awareness and expertise

2. Well-timed diagnostics

3. Simplified procedures for drug provision

4. Compliance with legislation in its entirety

5. Adequate control of drugs available in private pharmacies

6. Registration of more appropriate drugs

7. The establishment of the National Plan for Rare Diseases

8. Creating the registry of rare diseases

9. Other ----------

THANKS FOR YOUR COOPERATION!