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EVALUATION OF PHARMACISTS' KNOWLEDGE AND ATTITUDES REGARDING RARE DISEASES AND ORPHAN DRUGS

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Rare diseases (RD) are very heterogenic group of disorders affecting less than 5 out of 10.000 people in the European Union (EU), at the same time putting them in danger or disabling them chronically. It is estimated that only in Serbia almost half a million people suffer from some RD. In spite of rarity, they represent an important medical and social problem.

The aims of this pilot project were to evaluate the pharmacists` general knowledge and specific knowledge regarding RD, regulatory requirements and availability of drugs for the RD in the Republic of Serbia as well as pharmacists` attitudes and understanding of the health public importance of RD and drugs` availability. The prospective cross-sectional KAP study was conducted during 2012, on a convenient sample of the community pharmacists from the territory of the Niš branch of Pharmaceutical Chamber of Serbia. The questionnaire was fully completed by 139 pharmacists; 89.2% were females with mean age of 43.4±9.1 years. More than half of the respondents (66.9%) knew that there was no Register of RD in Serbia, but did not know the estimated percentage of the EU population suffering and the prevalence of RD (67%, 51.8%, respectively). Insufficient information about the problem points to insecurity in basic epidemiology and regulatory knowledge. The majority of the respondents supported the establishment of the regulatory instruments for the promotion of the research and development of the orphan drugs for RD. *Acta Medica Medianae 2013;52(2):23-32.*

Key words: *rare diseases, orphan drugs, regulation, pharmacists, KAP survey*

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Introduction

Rare diseases are an important medical and social problem which many health systems in both developed and undeveloped countries all over the world are facing (1). According to the definition proposed by the EU Committee of Experts on Rare Diseases, these are diseases that are detrimental to human health or disable it chronically with an occurrence of at least 1:2000, i.e. affecting less than 5 out of 10.000 people. National regulatory body defines the criteria for "rareness" of each disease, based upon its prevalence. Pursuant to the definition of the U.S. Food and Drug Administration (FDA), these are diseases with the prevalence of 1:1250 people (2). However, many rare diseases (>3500) occur only among few people all over the world (3). Differences between the prevalence of diseases and the population size make differences in the number of all rare diseases' patients as well as the number of specific rare diseases' patients in one country or region.

Rare diseases comprise a very heterogeneous group of disorders with little in common except the fact that they are rare. About 6-8.000 types of rare diseases have been listed in the world among which 80% can be attributed to genetic heritage, whereas the remaining ones are either contagious or autoimmune (4). According to the EU Committee of Experts for Rare Diseases, these illnesses affect about 6-8% of people in the European Union (about 30 million out 50 million people). A common characteristic is that these diseases are chronic and degenerative with the first symptoms occurring already at birth or during early childhood (with 50% of cases) and are often treated for entire lifetime if a known treatment can be applied. Further characteristics indicate that the most common consequence of rare diseases is permanent disability, loss of patient autonomy and decreased quality of life among affected patients (4). Yet, more than 50% of rare diseases may develop only at mature age, such as Huntington's, Fabry and Crohn's.

Orphan drugs are products used for diagnosis, prevention and treatment of rare diseases and as such they are necessary for the need of public health although their development has not been supported by the pharmaceutical industry due to economic reasons. From the historical point of view, health systems have not been dealing

much with the needs of patients suffering from rare diseases but have rather been directed to the most common diseases. Hence, the name of drugs used in treatment of these diseases was orphan drugs. The fact is that about 30% of all drugs labelled as "orphan" are classified as innovative drugs (3).

For many diseases there is no treatment and if there is some, provided it has started on time and that it is available, there may be a good prognosis that most of the affected people have normal life. The diagnosis and the treatment of those affected by rare diseases are difficult. A further difficulty can be identified as insufficient awareness and knowledge about these diseases among laymen and professionals. That is why rare diseases are also labelled as "orphans" (they have been neglected by professionals, researchers and the general public) (5).

Health policy creators and regulators do not recognize rare diseases as a public health problem because health resources and health technology are limited and directed towards the most common diseases most people in the world suffer from. This creates an even greater gap between the equity and accessibility of treatment to patients with rare disease and those with common diseases, because you should not expect mutual understanding among patients for treatment of others at the expense of other treatment. The pharmaceutical industry has no interest in the research and development of new drugs in this area, whereas health services, i.e. professionals involved in diagnosing and treating patients show little interest and inadequate understanding, especially since they have little knowledge of rare diseases. Rare diseases became a prevailing problem in the area of public health during the nineties in the previous century when the World Health Organization pointed out the need that this group of patients should be taken care of. Therefore, the interest of researchers, practitioners and the industry has been provoked (7, 8).

From the point of view of people in the health industry, it is very important to have adequate knowledge, training and valid information at hand so as to inform the general public in an appropriate way. For instance, 1,300 rare diseases have been described properly whereas other rare diseases have no adequate medical description. Doctors and pharmacists are not trained enough and they lack experience in the treatment of rare diseases.

A particularly sensitive issue in this area is the availability of medical services. The prices for orphan drugs are usually high. A comparative research conducted in 15 countries of the European Union showed that the availability and price clearly differ in each of these countries (9). Since the costs for orphan drugs are the same in both developed and undeveloped countries, it is very difficult for patients in undeveloped countries to afford such treatments. Due to the fact that the prices of some innovative orphan drugs, which can be successfully used for treatment of a few diseases (i.e. Gaucher's disease, Morbus Fabry), are very high, and that the state can burden the medical funds, these drugs are usually not registered by pharmaceutical industry but frequently donated to charity for the most difficult cases until the state accepts participation in the financing of treatment of patients (6). In order to make services available, a particular infrastructure is needed to provide medical and psychosocial protection. An adequate allocation of resources may considerably decrease the burden of the disease for many patients. A European research focused on diagnosing and accessibility of services within the health sector and social services in 20 European countries has shown that 40% of rare diseases were first diagnosed wrongly, which brought about severe consequences including inadequate and expensive medical interventions, such as surgery and psychological treatment, while 59% of the respondents said they had to either cut down on their professional activities or completely stop practising them because of their illness or because they had to take care of a family member with a rare disease (10).

From the point of view of health policies, regulations and legislation, orphan drugs differ from country to country so that authorizing sales and availability through health security is being regulated in different ways.

United States (Orphan Drug Act of 1983), Japan (1985) and Australia (1997) are far ahead with respect to regulations compared to the EU (2000). In both regions of the world, there is the same two-step system for the approval of regulatory authorities to market, by giving "marketing authorization for "orphan" drug (MA) or "orphan designation" and a clear incentive to industry research and development of this group of drugs (eg. fee reduction and exemption from various taxes, faster simplification of procedures for obtaining a medicinal product, obtaining an extension of the exclusivity of the product in the market) (6, 11). By the end of 2006, the FDA issued 1713 "designations for orphan drugs" and 304 MA, whereas in the EU 443 "designations for orphan drugs" were issued and 32 orphan drugs were licensed in the same period (11, 12). A growing interest in the research and development of orphan drugs has become evident as a result of the development of biotechnological drugs.

In the Republic of Serbia, among 1969 registered drugs with proprietary name from 2012-drug list of the Health Insurance Fund (Republicki fond za zdravstveno osiguranje – RFZO) 28 INN drugs from the Orphanet base were listed, specific for certain rare diseases (13). Interest in orphan drugs and rare diseases has grown since 2000. The National Organization for Rare Diseases in Serbia (Nacionalna organizacija za retke bolesti Srbije – NORBS) was founded in 2010 and according to their data, there are between 480,000 and 640,000 people in Serbia suffering from some rare disease (10);

however, despite such a huge number of patients, there is still no reference to national centre for the registration of rare diseases, there is no register of drugs for rare diseases nor is there a codebook of rare diseases.

The Regulation of Import Conditions for Drugs and Medical Equipment without a licence for Sales (2008) includes a list of 258 rare diseases in the Republic of Serbia (14). Pursuant to the Law of Alterations and Amendments of the Health Care Law (2011), patients suffering from some rare disease belong to a particularly sensitive category, classified into the group of patients exposed to a higher risk of illnesses potentially covered by insurance (15). Implementation, improvement and development of activities and evaluation programmes of health promotion, primary, secondary and tertiary prevention as well as the control of rare diseases were recognized by the Law of Public Health (Official gazette Sl. Glasnik RS, issue 72/2009) as a health activity of public interest (15). The current changes in the Health Care Law and Health Insurance in 2011 initiated the creation of the fund within the Ministry of Health of the Republic of Serbia to cover rare diseases. Most patients suffering from rare diseases in Serbia have not been recognized in the system of health insurance. They are facing stigma and high prices of drugs.

Aims

1. The evaluation of knowledge of pharmacists regarding rare diseases, regulations of drugs needed for the treatment of rare diseases and their availability in the Republic of Serbia.
2. The evaluation of the attitudes of pharmacists and their understanding of the public importance of rare diseases and the availability of treatment for patients suffering from rare diseases.

Methods

The study represents a pilot project conducted with an appropriate sample of pharmacists. The research was realized in pharmacies at the primary level of health protection during the year 2012. The prospective cross-sectional study included the population of licensed pharmacists on the territory of the Niš branch of FKS (mermbers from the Kosovo and Metohia were not available). The Niš branch with Kosovo and Metohia includes the following districts: Nišavski, Zaječarski, Borski, Rasinski, Pirotski, Toplički, Pčinjski, Jablanički, Kosovski, Kosovsko-pomoravski, Kosovsko-mitrovački, Prizrenski and Pećki. It comprises 770 active members from all parts except members from Kosovo and Metohija (17). For the data collection, a specially designed instrument was used according to the aims of the research. A questionnaire was designed by means of which the socio-demographic characteristics of the respondents could be collected. At the same time, their knowledge could be assessed and their attitudes could be estimated.

The questionnaire consisted of 11 multiple choice questions, two scales for the evaluation of attitudes and two problem-solving tasks by means of which the participants could express their attitudes to the importance of rare diseases as far as public health was concerned. A national study was planned and an improved version of this questionnaire was supposed to be used. The statistic computer processing programme used was SPSS (SPSS 18.0 for Windows, Inc., Chicago, IL, USA). During the data processing, the methods of descriptive statistics and correlation analysis were used.

Results

93.4% of respondents (170/182 pharmacists) completed and returned the questionnaire but the data basis for the analysis included only 139 completed questionnaires. The description of the respondents according to gender, age and level of education as well as work experience in a pharmacy is presented in Table 1.

A little more than half of the polled pharmacists used only one source of information while the remaining information regarding rare diseases was collected from various sources (Figure 1).

The respondent's opinions regarding the frequency of rare diseases in respect to a certain sample of population suffering from rare diseases are divided. Almost half of the respondents believes that rare diseases have an occurrence of 1 per 2.000 people, one third of them is aware of the situation regarding rare diseases (48.2%, 33.0% respectively), while the remaining number of respondents did not provide a correct answer, which is presented in Figure 2. Differences between the respondents knowledge about regulatory issues concerning rare diseases and orphan drugs are presented in Figure 3.

The respondents believe it is necessary and possible to have a regulatory instrument for further research of rare diseases. The results indicate that almost all respondents (91.4%) agree that it is necessary to have a regulatory instrument (law, regulation or agreement) which would support further research and development of drugs for rare diseases. However, when asked whether such regulatory instrument could be implemented, only 64.7% believed that such instruments could be implemented while one third among them (29.5%) was not sure about it.

The evaluation of the respondents' attitudes regarding the awareness of the importance of rare diseases in the public health sector, the availability of drugs and the encouragement for research and development of drugs, was evaluated by means of a five-level scale ranging from "very important" to "unimportant" and the results are presented in Table 2.

Only a very small percentage of respondents in the entire sample, 0.7 – 2.9% did not have any opinion regarding some of the attitudes.

The correlation analysis provided the relationship between certain variables based on the scale used, such as difficult economic situation of the family/ guardian in correlation with the lack of public awareness about rare diseases (r=0.535; p<0.01); the lack of access to existing efficient treatment because drugs have not been registered in the country (r=0.670; p<0.01); the lack of priorities in managing the needs of patients within the public financing sector (r=0.401; p<0.01); the difficulties in the area of research of specific diseases (unknown aetiology, lack of research material) (r=0.412; p<0.01; no investments in furthering research and development of drugs needed for rare diseases (r=0.376; p<0.01); no drugs for rare diseases (r=0.343; p<0.01); patients suffering from rare diseases may have the right to free treatment with some drugs even if the treatment itself is expensive (r=0.381; p<0.01).

The lack of public awareness in the society regarding rare diseases is in correlation with the lack of priorities in managing the needs of the people suffering from rare diseases in the public financing sector (r=0.526; p<0.01) and the attitude that the ones suffering from rare diseases may have the right to free treatment with some drugs even if the treatment itself is expensive (r=0.389; p<0.01).

The lack of access to existing efficient treatment, because drugs are not registered in the country, has been correlated with the following variables: the lack of priorities in managing the needs of people suffering from a rare disease in the public financing sector (r=428; p<0.01) and the lack of drugs for the treatment of rare diseases (r=0.411; p<0.01).

The distribution of rare diseases has been correlated with the difficulties encountered in the research of specific diseases (unknown aetiology, lack of research material (r=0.402; p<0.01) and the load of the disease itself (r=0.646; p<0.01).

Table 1. Basic demographic characteristics of respondents

Characteristic	Value	***p***
Total number of respondents	139	
Age (years)	$\bar{x}$ =43.4±9.1	*p*<0.01
Age range	24-72	*p*<0.01
Male	15 (10.8%)	*p*<0.01
Female	124 (89.2%)	*p*<0.01
Post-graduating training	12 (8.6%)	*p*<0.01
Work experience in pharmacy	$\bar{x}$ =16.7±9.8	*p*<0.01

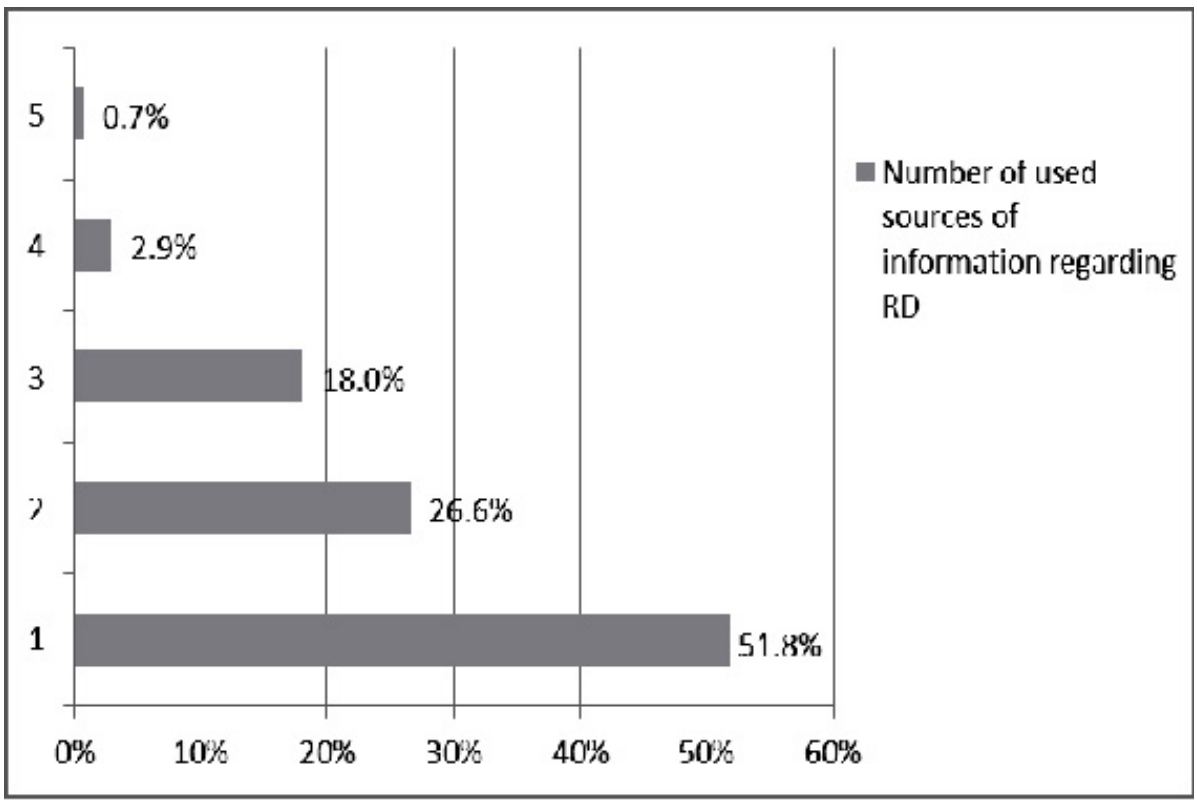

Figure 1. Number of used sources of information regarding RD

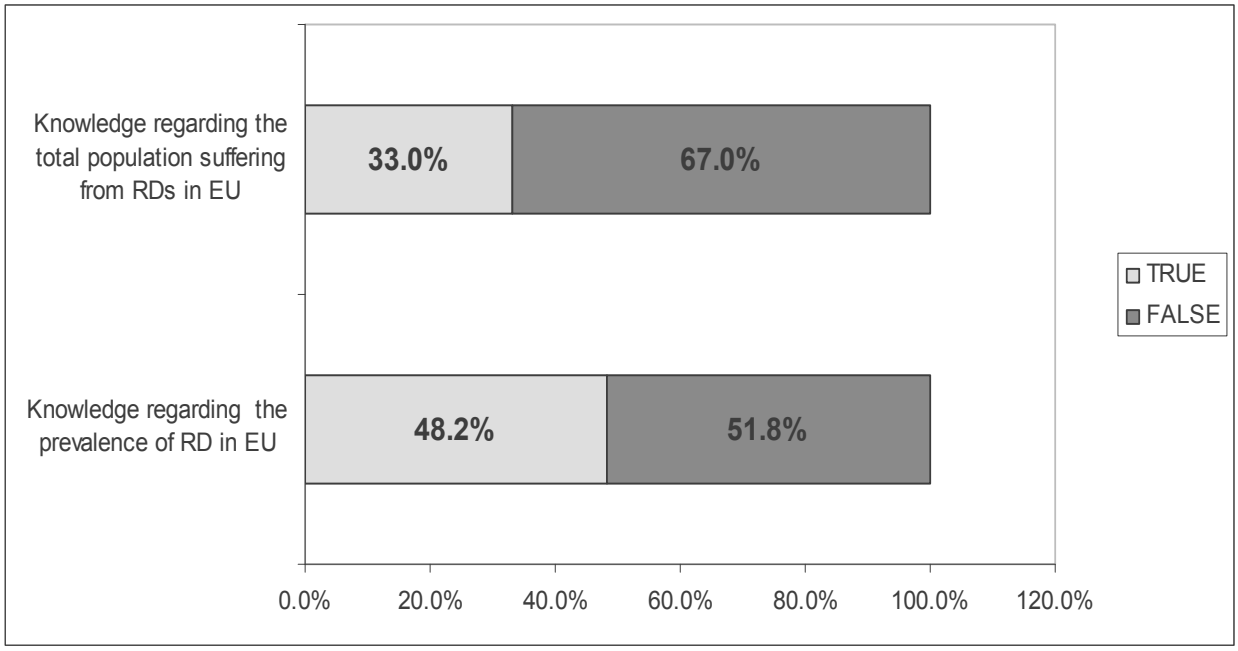

Figure 2. Knowledge regarding the total population suffering from RD and the prevalence of RD in EU

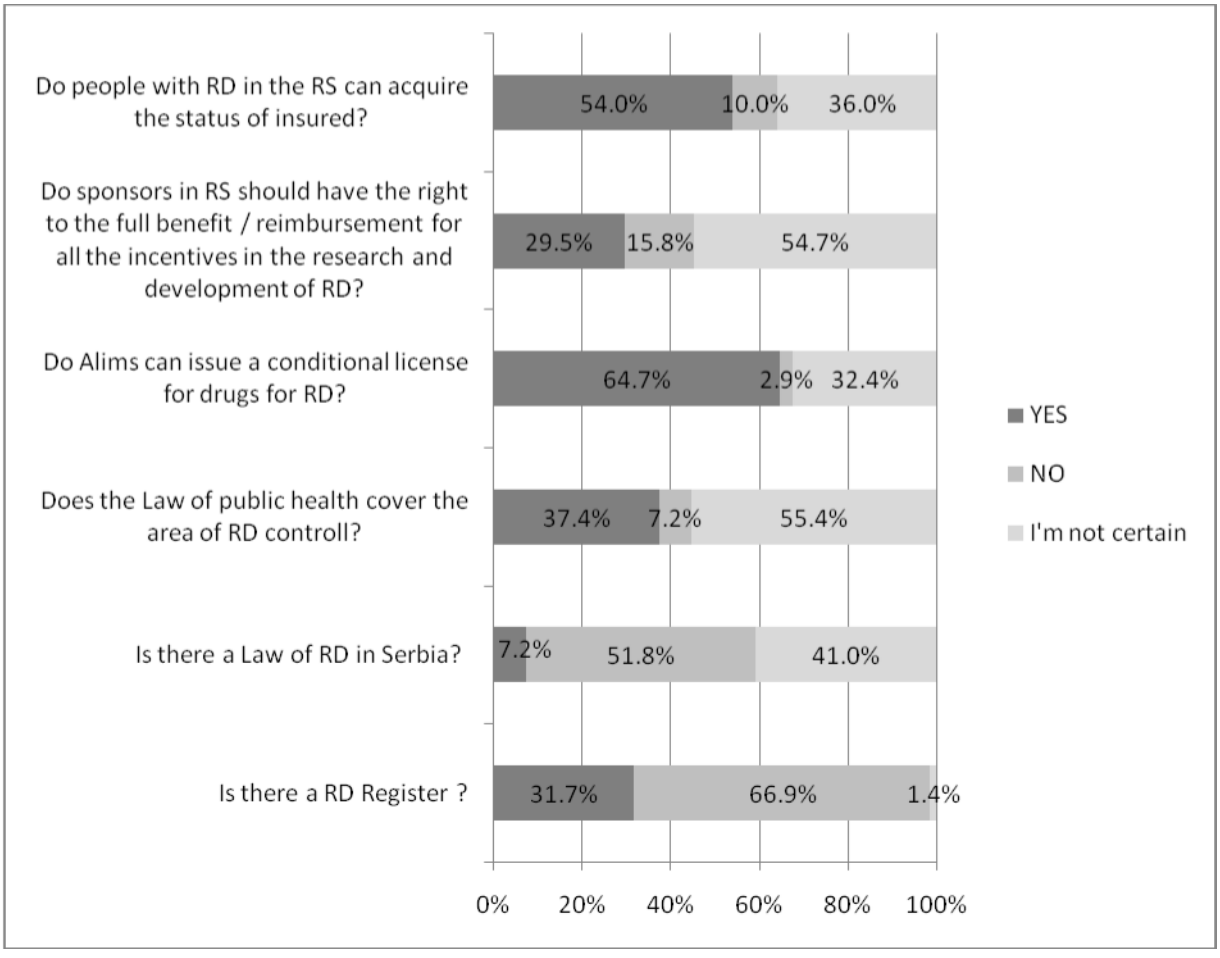

Figure 3. Level of knowledge among the respondents regarding orphan drugs and legislation about RD

Table 2. Pharmacists' attitudes regarding the importance of RD in the public health sector and the availability of treatment to patients suffering from RD

Items in the Questionnaire to assess pharmacists attitudes	Very significant (%)	Mostly significant (%)	Significant (%)	Little significant (%)	Insignificant (%)	No judgment (%)
Lack of public awareness of rare diseases	51.8	18	27.3	1.4	1.4	0
Threatened economic situation of the family / carers	71.2	14.4	12.9	1.4	0	0
Lack of access to existing effective treatment	69.8	18	11.5	0	0.7	0
No access to existing effective treatment	64	22.3	10.1	3.6	0	0
Prevalence of diseases	36.7	25.2	28.1	7.2	2.2	0.7
Burden of diseases	50.4	20.9	18	10.8	0	0
Diseases/ specific research difficultes (unknow etiology, lack of research material)	54.7	23	18.7	2.2	0.7	0.7
Lack of health needs driven priority setting in public funding	61.2	20.9	15.1	1.4	0.7	0.7
No drugs for rare diseases	61.2	25.2	10.1	2.2	0.7	0.7
Ineffective drugs for rare diseases	44.6	26.6	20.9	2.9	2.2	2.9
No adequate health delivery infrastructure and human resources in developing countries	51.8	25.9	18.7	2.2	1.4	0
Inadequate health delivery infrastructure and human resources in developing countries	54	25.2	19.4	0.7	0	0.7
No incentives for investment into research and development for rare diseases	54.7	28.8	13.7	0	1.4	1.4
Inadequate incentives for investment into research and development for rare diseases	54	24.5	17.3	1.4	0.7	2.2
No investment in research and development of drugs for rare diseases	57.6	24.5	14.4	0.7	0.7	2.2
Inadequate investment in research and development of drugs for rare diseases	57.6	23	13.7	1.4	1.4	2.9
Poverty as an important determinant of health	64.7	22.3	8.6	2.9	1.4	0
Poverty as a cause of the lack of treatment (no drugs in poor countries or drugs are not available, insufficient investment in research and development)	66.2	20.1	10.1	1.4	2.2	0

$p < 0,01$

Discussion

During the last few years, the world has witnessed an increase in interest of the health authorities as well as the public regarding rare diseases and drugs needed for their treatment. The number of studies related to the general knowledge of healthcare employees and the evaluation of their attitudes is limited whereas the results of most of this research are not homogeneous and there are considerable differences between the groups included in the research. For the purpose of this research conducted among pharmacists, two European and one national study was used: one was conducted among the general public (17) and the other among healthcare employees, which included pharmacists from the pharmaceutical industry (Delphi) (18) and the study conducted by Mitev including healthcare employees in Bulgaria (19).

A good response of respondents in this research may indicate both an interest of healthcare employees in this area as well as the fact that the data were collected with an anonymous questionnaire.

Almost half of the surveyed pharmacists in our research (48.2%) knew about the correct prevalence of rare diseases, which is a much higher percentage in comparison to the results indicated in the Bulgarian study with doctors (19.82%) (19).

This research showed that 33% of the respondents provided a correct response to the answer regarding the range of the European population suffering from rare diseases, while a rather lower number of respondents (6.9%) supplied a correct answer to that question in the Bulgarian study conducted by Mitev (18). The differences noticed about the knowledge in both studies can be attributed to the different instruments used because of the Bulgarian study, Mitev used a telephone interview, which implies less time for the respondents to check some other sources of information (18). There are huge differences in the knowledge among the respondents in the Serbian research regarding regulating aspects of rare diseases and drugs used for the treatment in the Republic of Serbia because pharmacists supplied correct answers to 6 questions with answers ranging from 15.8% to 66.9%. The lowest level of knowledge was shown by pharmacists with the questions not directly related to their everyday work, such as the registration of drugs used in the treatment of rare diseases and the Law of Public Health (15.8%, 37.4% respectively). When answering the questions related to their everyday work activities, the pharmacists provided mainly correct answers. A large percentage of respondents knew about "the conditional licence for the registration of drugs used in the treatment of rare diseases" (64.7%); they knew that there is no register of rare diseases (66.9%), the possibility of the sick to pursue their insurance in our country (54%) as well that there is no national Orphan drugs' Law (51.8%). The available literature does not provide any information regarding the knowledge of regulatory laws among healthcare employees so that such information is invaluable.

In the second part of this research, which deals with the attitudes of pharmacists about the importance of public health importance of rare diseases and treatment availability, the result of two studies, one conducted among the general population (17) and the Delphi research conducted among healthcare employees including pharmacists from the pharmaceutical industry (18) can be compared.

Almost all the respondents in both the Serbian and the Delphi research agree that it is necessary to have regulatory instruments (law, regulations or agreements) so that research and development of rare diseases could be furthered. At the same time, they are careful when predicting the feasibility of implementing such instruments (18). Such carefulness expressed by healthcare employees can be interpreted based on their attitude regarding awareness and the long-term effects such changes would ask of regulatory bodies within the current legislation while related to the furthering of research and development of financing sources for this group of drugs. Almost the same number of respondents in this and a similar research conducted in Europe in 2010 agrees upon the attitude that public awareness of rare diseases is still missing (17). Providing health support to people suffering from rare diseases and a complete compensation of costs for some drugs, even if they are expensive, was recognized as an an important measure (17). Agreement with the idea that drugs for these diseases are completely refunded is present in both this and the European research. As far as the attitudes regarding the lack of priorities in managing the need in the public health financing sector are concerned, it was established that it was one of the most important influence on the treatment of the sick. Both researches agreed on this issue but some differences between the Serbian and the European studies could be established in the attitudes among respondents regarding different regions in Europe.

This research showed that 9 out of 10 pharmacists (97.2%) identifie the lack of priorities in the management of the needs of people suffering from rare diseases as important within the public health financing sector, which may be interpreted as a high level of awareness among the respondents. Similar attitudes among healthcare employees were not measured in the European studies but an interesting fact in the general public could be identified – 39% of the respondents in the European study believe that the priority in their countries regarding health protection can be attributed to other health problems and not rare diseases. This points to the fact that broader and further research is needed in the population of healthcare employees who could influence the decision making within healthcare policies by means of their higher awareness and attitudes (17).

The attitudes of respondents regarding the treatment of rare diseases as far as existing drugs or their unavailability is concerned, since they are not reregistered, were evaluated in a similar way in this research and the research conducted among the population of healthcare employees (18). In the Delphi research, 41.0% of respondents believed that the lack of or difficult accessibility to existing efficient treatment is important while the Serbian research showed that 69.8% of respondents believed that the lack of accessibility to existing efficient treatment is very important. A total of 64% of the respondents in the Serbian research believed that the lack of accessibility to existing efficient treatment is very important.

The attitudes of the respondents regarding the treatment of rare diseases related to the lack of drugs or their unavailability as they are not registered were evaluated in a similar way in this research and the Delphi research. While 58.3% of respondents in the Delphi research believed that the unavailability of treatment due to a drug missing or not being efficient was important, the Serbian research showed that among the pharmacists, 61.2% believed the missing drug in the treatment of rare diseases was very important and 44.6% of the respondents believed the unavailability of treatment was very important (18).

The lack of investment or inadequate investment in the furthering of research and development of drugs of rare diseases was considered important by half of the respondents in the Serbian research and by almost half of the respondents (45.2%) in the Delphi research (18).

Poverty as a very important determinant of health and the cause of missing treatment, in the Serbian research, was attributed to a high level of importance, a slightly higher level than in the Delphi research. The reason for this type of attitude in the Serbian research may be found in the difficult economic situation and general poverty in Serbia (20).

Inadequate distribution of infrastructure and resources in the health sector in undeveloped countries is of huge importance according to half of the pharmacists in the Serbian research and almost the same number of respondents agrees in the Delphi research (18). Apart from that, the lack of drugs for the treatment of rare diseases in the Serbian research was rated as highly important (very important by 61.2%, mainly important by 25.2%, and important by 10.1%). For 30% of respondents in the Delphi research, the lack of drugs for rare diseases is very important. 58.3% believe that this issue is important because of the lack of treatment of rare diseases (18).

Similarly, the respondents in the Serbian research chose the issue of financing as very important. The lack of an adequate distribution of infrastructure in the health sector as well as the professional workforce in undeveloped countries was considered important by 51.8%, while 25.9% in the Serbian research rated this as mainly important. In the similar Delphi research, 45.9% rated this issue as very important and 41.0% as important (18).

The issue of financing was a high priority in both researches. The lack of investment in the research and development of drugs for rare diseases, in the Serbian research was rated as very important by 57.6%, mainly important by 24.5% and important by 14.4%. In the Delphi research, 48.4% rated the lack of investment in the research and development of drugs for rare diseases as very important, 48.4% agrees with this and 37.1% think this issue is important (18).

The similar attitudes in the Serbian and the Delphi research indicated the complexity of these issues and the need to support a strategy which would raise awareness, decrease poverty, include rare diseases into the priorities of research and development, improve and sustain financing, promote health and the research infrastructure in all countries. Most respondents in the Serbian research supported the implementation of regulatory instruments for furthering the research and development of drugs for rare diseases. One of the key issues is the national responsibility for financing research and development, which is considered necessary by the most respondents in the Serbian study. That is why it might be of interest to spread and direct research in the area of determining the measures, which could contribute to future instruments needed for promoting the research and development of rare diseases' treatment.

Conclusions

The general knowledge of pharmacists regarding rare diseases, regulations and drugs for the treatment of rare diseases in public pharmacies on the territory of the Niš branch FKS is in accordance with the knowledge about rare diseases identified in European studies. The lack of knowledge among pharmacists regarding the problem of rare diseases indicates a high level of insecurity when it comes to knowing about the basic regulation demands related to rare diseases and the drugs for their treatment in Serbia. However, based on the answers to the questions regarding pharmacists' attitudes related to rare diseases being a public health problem, a general attitude of interest in this problem is obvious. The similarity between the responses in both the Serbian and the European research indicates the complexity of these issues and the need to support a strategy which would raise awareness, decrease poverty, and include rare diseases in the priorities of research and development of infrastructure in all countries. The encouragement to develop effective treatment of people suffering from rare diseases may be found in the non-economic social values, i.e. the wish to develop a fair approach to the treatment of the sick. The basic message conveyed by this research as far as the creators of the healthcare policies in the Republic of Serbia are concerned might be that among the pharmacists in public pharmacies on the territory of the Niš branch FKS, a widely spread readiness could be noticed. This readiness is related to the wish to provide support to people suffering from rare diseases, within the range of professional possibilities. Furthermore, the readiness is related to the need for further and broader research, including pharmacies in certain campaigns or determining days for rare diseases as well as raising the level of general knowledge on rare diseases, regulations and the availability of orphan drugs.

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PROCENA ZNANJA I STAVOVA FARMACEUTA O RETKIM BOLESTIMA I LEKOVIMA ZA RETKE BOLESTI

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Retke bolesti su heterogena grupa životno ugrožavajućih ili hronično onesposobljavajućih oboljenja, koja se prema kriterijumima evropske regulative javljaju sa učestalošću od najmanje jednog prema 2000 stanovnika. Uprkos tome što su retke u populaciji, one predstavljaju važan medicinski i socijalni problem sa kojim se susreću zdravstveni sistemi širom sveta. Procene su da samo u Republici Srbiji od retkih bolesti boluje skoro pola miliona ljudi.

Ciljevi ovog pilot projekta bili su da se procene opšta i specifična znanja farmaceuta o retkim bolestima, regulativi u vezi sa lekovima za lečenje retkih bolesti i njihovoj dostupnosti u Republici Srbiji i utvrde stavovi farmaceuta o značaju retkih bolesti i dostupnosti terapije za bolesnike.

Prospektivna studija preseka obuhvatila je farmaceute koji rade na teritoriji niškog ogranka Farmaceutske komore Srbije. Prikupljanje podataka obavljeno je tokom 2012. godine pomoću strukturiranog, anonimnog upitnika, posebno konstruisanog prema ciljevima istraživanja, a za obradu podataka korišćene su metode deskriptivne statistike i korelaciona analiza.

Upitnik je potpuno popunilo 139 farmaceuta, pretežno ženskog pola (89,2%), prosečne starosti 43,4 ±9.1 godine. Više od polovine ispitanih farmaceuta (67%) ne zna da 6-8% populacije u EU boluje od neke retke bolesti, dok 51,8% smatra, što je pogrešno, da je prevalenca u Evropi manja od 5 na 10 000 ljudi. Većina ispitanika (66,9%) zna da u Srbiji ne postoji Registar retkih bolesti. Nedovoljna informisanost farmaceuta ukazuje na nesigurnost u poznavanju osnovnih regulatornih zahteva u vezi sa retkim bolestima i lekovima za njihovo lečenje. Većina ispitanika se u svojim stavovima zalagala za uspostavljanje regulatornih instrumenata za promovisanje istraživanja i razvoja lekova za retke bolesti. *Acta Medica Medianae 2013;52(2):23-32.*

Ključne reči: *retke bolesti, orphan lekovi, regulativa, farmaceuti, KAP studija*

