

Health systems sustainability in the framework of rare diseases actions. Actions on educational programmes and training for professionals and patients

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Abstract

Rare disease community is one of the largest patient populations in the world estimated to be 350 million of people. Collectively common, rare diseases pose a significant medical and economic burden for health systems worldwide. In this respect, rare diseases are considered a priority of public health. The study is a review aimed to determine whether there is evidence that education plays a key role in building sustainable health system and will allow better health and well-being for people with rare diseases to be achieved. This review shows evidence that providing quality education through different ways and actions the lives of people suffering from a rare disease and their families can be improved.

Key words

- education
- health care system
- rare disease
- sustainable development

INTRODUCTION

According to WHO, a good health system delivers quality services to all people. Good healthcare services are those which deliver effective, safe, quality personal and non-personal health interventions to those who need them, when and where needed, with minimum waste of resources [1].

Nowadays, health care systems in both developed and emerging economies face the problem of insufficient resources to meet the anticipated health care needs of all citizens. Ageing populations, longer life expectancy, increasing demand for services, new technologies and new medicines all contribute to the financial pressures. Doctors and patients are aware of these pressures [2, 3].

In the context of concerns about rising health care costs and the lack of availability of necessary supplies required to maintain life, or a specific quality of life, rare diseases are drawing attention to the demand for special services, challenging whether patient expectations are realistic, and whether current models of service delivery are sustainable [4, 5].

Research based on decades of experience in the developing world has identified educational status (especially of the mother) as a major predictor of health outcomes, and economic trends in the industrialized world have intensified the relationship between education and health [6].

MATERIAL AND METHODS

To collect evidence that education plays a key role in building sustainable health care system for persons with RDs, an electronic literature search was conducted using different medical databases and additional information resources (including government documents, reports from international bodies such as the World Health Organisation, Eurordis or UNESCO and academic studies). The key search terms were education, health care system, sustainable development and healthcare professionals and rare diseases, from 2000 through January 2018. Studies reporting about education for healthcare professional who provide medical services to both children and adult patients with RDs or other diseases were included as well. Articles of interest were reviewed to determine which were relevant and subjected to analysis. Selected papers were later used to compare healthcare education between the patients with RDs and other disease groups to emphasize the need for specific and complex actions on educational programs and training in the field of RDs.

RESULTS

The facts – what is already known about rare diseases

Rare diseases (all together) represent an international public health issue and no one country, no one continent alone can solve the problems posed by rare diseases [7].

People with a rare disease (RDs) deserve equal access to care, treatment and rehabilitation as the other patients while the kind of disease they are suffering from put them in a special relation with their normal legitimate aspirations and with the rest of the society. Moreover, 50% of RDs affects children who have difficulties in attending school and integrate themselves into local activities of community [7].

Prices for RD healthcare services are rising at a rate that is unsustainable for most patients and their families due to chronic illnesses, development of expensive new medicine, high-tech medical equipment and procedures for genetic diagnostic testing, patients expectations for access to the latest technologies. Therefore, they need a healthcare system dedicated to their specific needs and able to assure a coherent approach to their specific problems [8]. But the diagnosis and treatment protocols have to be balanced with resources conservation and management [9].

The configuration of health services varies from country to country but everywhere education and training of professionals are an important component of the sustainability of health system.

Within this context it should be ensured that sufficient health professionals are trained, competent, with the necessary knowledge and skills. Likewise, strong links need to be made in both directions between education providers and Centers of Expertise for rare diseases.

When dealing with rare diseases, medicine is sustainable only if:

- medical treatment is meant not only to improve the length of the patients' life but the quality of their life as well;
- the life quality improvement of the sick people does not hurt the quality of life of the healthy people;
- the adequate medical and social services provided in order to meet these needs presently remain available for the future generations [4].

For reaching these three targets one needs a pragmatic farsighted visionary educational system with a holistic perspective. The four pillars of education [10], "learning to know, learning to do, learning to live together, learning to be" should be applied to change the health knowledge, skills, awareness and health attitudes and behavior regarding sustainability in the health care system and the connection between health and the environment.

DISCUSSION

"The great aim of education is not knowledge but action"
(Herbert Spencer)

We know rare diseases exist. But it's not enough to know. We need to act based on that knowledge. There is a difference between what we know, learn and what we do. Education should bridge the gap between knowledge and behavior informing, training and motivating people (professionals, parents, caregivers, communities) to translate the health knowledge into actions. Therefore education can serve as a powerful tool in transforming rare diseases care. Worldwide education is an important mechanism for enhancing the health and well-being of individuals because it reduces the need

for health care, the associated costs of dependence, lost earnings and human suffering [11]. According to Eichler et al., 2009 [12] on the health system level, the additional costs of limited HL range from 3 to 5% of the total health care cost per year and on the patient level, the additional expenditures per year per person with limited HL compared to persons with adequate HL range from US \$ 143 to 7798.

Having in mind these challenges, education policy-makers and stakeholders have to rethink the strategy in the age of precision medicine characterized by changing of the paradigm of health care from being reactive to being proactive [13]. Patient is a person, not just a diagnosis. In addition, conventional medicine could not to solve all problems of persons with RDs. A new strategy directed to holistic and integrated care is the hope and future for RDs. The strategy will determine where they focus their attention over the long term: to develop healthier habits, incentivize healthier consumption and develop an environment and infrastructure that facilitate population health [14]. Educational actions should be implemented in a way that planning, delivering and monitoring Rare Disease issues become core work of the health system. A sustainable health system promotes, maintains and improves the health of population.

We have chosen the following actions on formal and non-formal education for a sustainable health care system in the framework of RDs hoping to have a real impact on RD patients life.

Integration of an education package on rare diseases in the curriculum of the medical degree course for professional development – students are the future for RD patients and their families

Being rare and complex, RDs should be approached holistically. Therefore a collaborative and multidisciplinary health and social care team should be involved in the management of patients with RDs. At the moment, the number of healthcare experts in rare diseases is low. Special undergraduate and postgraduate education and training programs should be developed and supported in the field of rare diseases.

Coping with rare disease from a medical care point of view requires professional creativity as well as the capacity to integrate new concepts and therapies within the old state of knowledge. This could be achieved only if the training of the medical students include more than updated information, the necessary ingredients for the development of an individual professional personality characterized by scientific and moral convictions, based on which a dynamic and proactive behavior in coping with the rare diseases challenges could be built. That would require appropriate curriculum, pedagogy, tools of assessment as well as the adequate overall structures [15]. Medical students should be encouraged to participate in programmes designed for RDs. In addition, universities and education providers should create opportunities for medical students to learn about RDs and understand patients experience, keep them in contact with RD Centres of Expertise and provide them links to patient organizations (Eurordis, Nord) and reference portal/knowledge databases (Orphanet).

Continuing Medical Education and training programmes for all healthcare professionals, allied health professionals and industry professionals

Learning is an ongoing process and by offering continuing education and training, universities and other accredited medical centers are committed to provide best resources and training for all healthcare professionals who are encouraged to take advantage of these opportunities. Continuing education programmes on RDs for health professionals support an educated and informed health workforce to provide the best health services to populations worldwide and to achieve the best possible health for all [16]. A variety of courses and training modules (live or online events) on RDs topics is offered for physicians, nurses, dentists and other healthcare professionals aiming to improve medical practice and implement holistic patient care. In addition, CME facilitates lifelong learning and is a fundamental factor in the maintenance of certification.

Integration of healthcare sustainability concept in medical schools and universities – from basic to higher education

To respond the needs for a sustainable health system one has to put a special accent on the formative rather than on the informative dimension of knowledge transferred to the future experts to be involved in the rare diseases field. That will allow the experts to adapt themselves to the rather fast progress of the science in this field. “Promoting health, promoting sustainable development emerges as a metaphor used by WHO, UNESCO and UNICEF for launching a new initiative “Make every school a Health Promoting School”. This makes schools a unique setting for preventive interventions, and school years an important period to establish healthy behaviours that will contribute to a lifetime of health promotion. However, challenges remain. Global mortality and morbidity estimates in children and adolescents suggest that school age children have significant needs for health promotion, prevention and health care services [1]. This concept is also applicable for RDs: approximately 50% of people affected by rare diseases are children and 30% of them will not leave to see their 5th birthday.

Involvement in rare diseases research activities

Rare diseases are a priority area of research funding for the European Union. Research activities, extending from bench research, clinical research to translational research, have the potential to develop attitudes towards science and potential science-based career. The future experts, while in school, must be integrated in the scientific research effort in the field of rare diseases. This could be done either by including them in different research programs or by inviting researchers to regularly inform the students on the priorities in their fields, as well on the obstacles they have met and on the ongoing achievements. Not only the professionals but the entire society must build upon the present scientific discoveries and respond adaptively to the new scientific ones, while assuring an intrinsic technologic dynamism able to maintain the viability and resilience of the present health systems’ best practices and performances within an ever changing societal environment.

Practical clinical training in centers of expertise (CEs)

CEs provide education and training to healthcare professionals from all disciplines, including paramedical specialists and non-healthcare professionals (such as school teachers, personal/homecare facilitators) whenever possible [17].

The medical students must be kept in permanent contact with the activities of the centers of expertise and diagnosis, thus developing their practical expertise. There are rare diseases of course, but there are individual patients, as well. One should develop appropriate expertise from both points of view.

Academic training providing with general scientific knowledge is extremely important, but equally important are the good practice guidelines, case studies and the understanding of the specific problems the practitioners in the RD concrete centers of diagnosis and treatment are coping with.

Development of the capacity to interact with patients and their families through education

Beside the medical training, the development of the social sensitivity and of the capacity to interact with the patients and their families would be crucial. In many cases the medical experts are able to deal with the challenges of the diseases but not equally able to deal with patients and their families, in order to put in place an early warning system which would allow for a medical intervention in an optimal time or for the necessary cooperation between the health system officials, the patients, the patients’ families and the whole society within which the patients must be treated, hosted and integrated. The school should train the all healthcare providers (primary, nursing or specialty care) for all health and social care services (preventive, curative, rehabilitative or palliative) from these points of view as well.

IT skills and knowledge acquired through professional training

There could not be put in place a national health system for rare diseases without a solid international and transnational medical, scientific, social and financial cooperation [18]. In today’s fast-changing health care environment, communication and collaboration can develop tailored and adaptable solutions that work, especially due to the complexity of rare diseases. Moving patients from home to one health care setting or to another can have negative effects on the health of patient and the well-being of family caregiver. Educational programmes develop the ability of healthcare professionals to change routine, adapt and evolve in order to manage rapid changes in the healthcare practice. The programmes include knowledge and skills related to information and communications technology (ICT) as well. In 21st century technology has changed the healthcare – information is moving and patients stay home but not “alone”. Therefore healthcare providers should be familiar with medical technology dealing with the storage, retrieval, sharing, and use of health care information, data, and knowledge for communication and decision making.

Whether or not health professionals go on working

together in partnership, through social media, such as LinkedIn, Facebook, Twitter or other social networking websites, they are connected to each other and can share personal experience and collaborate online on difficult rare disease cases.

Sharing and developing best practice in medical education and training

Best practices must be known and disseminated. Sharing examples of best practice in rare disease cases which worked well, can help medical schools and universities which educate students and train doctors to dive up healthcare standards. Free and easy access to the scientific and technology progress must be allowed among professionals. A strong health information infrastructure, when coupled with an effective health communication system, can improve health systems in drastic ways. For instance, European Reference Networks for rare diseases (RD ERNs) are foreseen by the Cross-Border Healthcare Directive to share knowledge, facilitate the mobility of expertise, and allow Member States to provide highly specialised services of high quality for patients where this would have been impossible without European networking, such as in the case of rare diseases [19].

For the success of educational programs dedicated to healthcare professionals working in the field of RDs additional supporting actions are needed such as:

Informing and educating the large and diverse public – raise necessary awareness, understanding and action among general population. In the field of rare diseases it is important to know but also to explain. Public awareness is crucial. The rare diseases cannot be cured or cannot be cured completely only in hospitals and at home. The people suffering of rare diseases must leave and receive appropriate treatment in society without scaring it or being rejected by it. A special public information and education to make RDs more visible it is therefore necessary. It is not only for the non-governmental organizations, social activists and administrative institutions to provide such public awareness. The physicians themselves must be prepared for being social activists and providing public awareness. Such abilities must be trained in the university alongside with the medical training. Public health campaign is required to disseminate the health education among the general population. It is known that the local and international media play a vital role as the link between health workers and the general public - “it has been observed that the greater the media emphasis on a given social theme or topic, the greater the public concern about the topic” [20]. Health authorities educate and entrust the media with essential health care information about people living with a rare disease, which is then relayed to the public in readily accessible formats through a variety of media channels [21]. Mass media campaigns have generally aimed primarily to change knowledge, awareness and attitudes, contributing to the goal of changing behaviour [22]. For instance, Rare Disease Day annual campaigns make the patients' voices heard and increase visibility of RDs at international level. At national level, RONARD (Romanian Alliance for RD) created an effective dissemination strategy for raising awareness, un-

derstanding and action in the general public. RONARD launched since 2011 an annual one-week school for medical journalists in the hope that three effects might occur: the learning of correct health information and knowledge, the changing of health attitudes and values, and the establishment of new health behavior. Medical journalists are an action and advocacy group in the position to influence and change the public health opinion.

For rural communities populated by people with limited health education via radio and television can be used as health communication tools. The programs should be planned to help them to stay healthy and to empower them to manage their health [20]. For instance, RONARD created NoRo Radio, a private radio station which disseminates scientific and health information of general / particular public interest on rare diseases and promotes the social integration on people with rare diseases as well. Printed media and Internet can be also used as health communication tools.

Education for general social support in the community. Rare diseases are chronic diseases which need long-term care and tend to require high-cost care. A sustainable health care system requires constant and sometimes massive financial funding. This implies the governmental budgetary contribution and private charity. Both must be mobilized. Moreover, the public contribution is to be facilitated by public understanding and acceptance. In other words, the society should accept that the public budgets must include financial provisions in support of the rare diseases treatment. The universities must prepare their experts in order to act as successful fund raisers as well, and to act within the society and in direct contact with the governmental competent bodies for getting and assuring sustainable financial resources in response to the rare diseases ongoing necessities.

Patient empowerment and engagement through education. There are different views on patient empowerment. But speaking about rare diseases as chronic conditions, in order to achieve good results, the patients need to be involved in the health care solutions [23]. There is evidence that good control of a disease is seen to be dependent on the quality of information the patient has received. Thus education must be tailored to the individual and provided in the context of patient's life [24]. Through education process patients acquire knowledge, skills, and self-confidence in controlling one's life and claiming one's rights. However, there is another view which regards patient empowerment. Rare disease patients are often the leading “experts” in their diseases – sometimes, for very rare diseases, they turn out to be the sole experts. Their knowledge and experience are valuable assets that are, thanks to empowerment, a driving force to shape rare disease related issues [25].

CONCLUSIONS

Our review provides a starting point and an evidence base to plan and deliver future educational programs for healthcare professionals, patients living with a rare disease, parents, caregivers, communities. Giving knowledge, training competences and promote active participation and responsibility through education, a new dialogue between policy-makers, stakeholders,

professionals and rare disease patients community will provide public health responses to special healthcare needs of people with rare diseases.

Authors' contribution

All authors were co-leaders in the conceptualization of the study, data analysis and interpretation, manuscript preparation and revision. All authors read and approved the submitted version of the manuscript.

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