#02/2014
ISSN 2299-1247

MEDICINE & PHARMACOECONOMICS JOURNAL

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JHPOR | Journal of Health Policy & Outcomes Research

INDEX COPERNICUS
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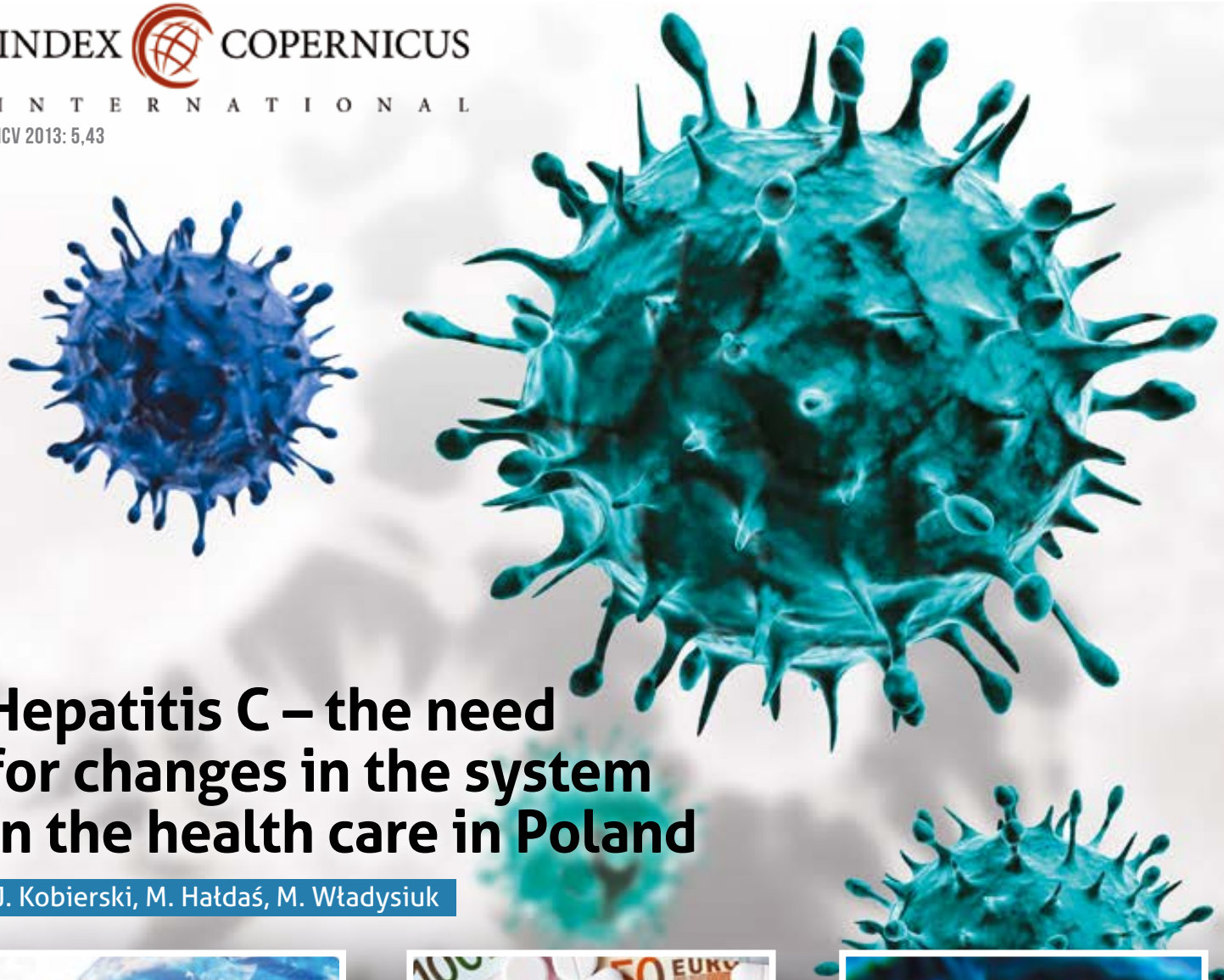
ICV 2013: 5,43



Hepatitis C – the need for changes in the system in the health care in Poland

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Do we need real world data Polish ISPOR Chapter Therapeutic Programs, Pharmaceutical Care and Pharmaceutical Law Section (TPPCPL) initial discussion

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Keywords:
real world data, registries, RWD

DOI: 10.7365/JHPOR.2014.2.10
JHPOR, 2014, 2, 94-99

ABSTRACT

Objective: **to discuss potential areas for use and sources of real world data in relation to medical treatment.**

Methods: **we performed Pub-Med and an internet based search in literature for definitions, sources and examples of real world data use.**

Results: **we identified 13 publications fitting our search criteria and we found that there are not many full publications related to real world data even if they can provide significant additional information regarding patient related outcomes, resources use, costs and the effect of therapies in a non-controlled environment and on broader populations than those from randomized clinical trials.**

Conclusion: **Real world data can provide additional information in relation to medical treatments however there is a need to focus attention on methodology of data collection, data quality, potential sources of data and ensure proper legal environment for data collection. Unfortunately, despite large number of publications identified in PubMed database, there are only few available in a form of full text papers, the majority of publications are only abstracts from scientific conferences. In the future in order to share RWD experience it will be of benefit to encourage authors to work on full publications and not to limit their work only to abstracts.**

INTRODUCTION

Nowadays it is not enough to obtain information related to new treatment option only from the randomized clinical trials. We can observe that the RCTs considered till now as the golden standard of evidence are not able to provide us with all the information we would like to have. Information about what the effectiveness of the new therapeutic treatment is, how it works on daily basis, if it is safe, what the real resources are used when treating patients with the new option. That and much more questions are related to real life data.

Objective: Our aim at the Therapeutic Programs, Pharmaceutical Care and Pharmaceutical Law Section (TPPCPL) task force was to investigate what is understood as real world data and what are the potential areas for use of real world data in relation to introduction of new therapeutic options to the market. This is only an initial approach to potential further discussion to be continued by the task force.

DURING THEIR WORK THE TASK FORCE MEMBERS LOOKED AT RWD SOURCES AND TYPE OF OUTCOMES (ECONOMIC, PROS AND CLINICAL OUTCOMES).

Methods: The analysis we performed was based on a literature search using the Internet. The Medline-PubMed databases have been reviewed. The initial search was focused on "real world data" term. Second search was restricted to real world data and the use of definition in the performed studies. Search strategy was based on terms: "real world data" [All Fields] AND "definition"[All Fields]. Reviewing all obtained publications from the performed search we analyzed in detail the publications from the period between years 2011-2014 and species – humans.

Results: We identified 7208 publications, however only 13 were meeting our search criteria (diagram 1). After analysis of the full texts we found 1 publication was the ISPOR Task Force report[1], 4 publications related to outcomes, disease or therapies[2,3,4,5], 1 was a quality of life study[6], and 1 was a methodology discussion paper in relation to statistical testing for clinical effectiveness studies[7]. Additionally, we realized that there are many abstract publications available. Based on those publications we found that real world data can provide additional valuable information to clinicians, payers, industry, patients and society on how the product or technology works in real life setting, in a non-controlled environment, outside randomized clinical trials; what the health related outcomes are, resources used for a disease treatment or the costs.

An important initiative identified was the one by ISPOR organization who has created a task force to discuss using RWD for coverage and payment decisions[1]. The task force defined RWD as data that is collected outside conventional Randomized Controlled Trials (RCTs). The real world data are raw data, non-informative while real world evidence is information that is organized to inform a conclusion or a judgment, it is typically undertaken according to a research plan and it is shaped to be informative, i.e. to clinical or payer decisions.

During their work the task force members looked at RWD sources and type of outcomes (economic, PROs and clinical outcomes).

They concluded that "Real world data are essential for sound coverage, payment, and reimbursement decisions. The types and applications of such are varied, and context matters greatly in determining the value of a particular type of evidence in any circumstance. Different study designs can provide useful information in different situations. Randomized controlled trials remain the gold standard for demonstrating clinical efficacy in restricted trial setting, but other designs—such as observational registries, claims databases, and practical clinical trials—can contribute to the evidence base needed for coverage and payment decisions."[1].

E.T. Masters in her publication about outcomes assessment and RWD related to pain mentions the different sources of real world data we have such as supplements to RCTs, large simple trials, patient registries, administrative claim databases, surveys, electronic health records[2].

Depending on the further data use and the sources for data which are available in each of the countries in the literature we can find examples of different approach to real world research and analysis. Some countries have disease registries in place and due to ongoing collection of real world information they are able to analyze the data about the disease and its treatment.

An interesting finding in our search was that despite a large number of records identified there are not that many real world data full text publications related to pharmaceutical products, registries or methodologies. Based on the final number of 13 full publications only 3 were related to collection of real world data using data from registries or electronic databases either in a prospective or retrospective way[3,4,5].

Based on the few identified publications and the large number of abstracts we can see cases when the real data can provide additional information on the effect of treatment, also on real length of the treatment[8] or how utilizing real world data can provide input into medical costs or health care cost reduction[9,10,11,12], and also examples showing how RWD can be useful to provide more understanding about disease burden, epidemiology or resources used in relation to the disease treatment[13]. RWD are not only valuable to learn more about treatment but can also provide significant input into prevention, information about the management of diseases like e.g. management of cardiovascular risk in primary prevention[14].

Important are the discussions about methodology for data collection, quality of the data and usage for decision-making. A discussion about methods how to enable implementation of RWD into network meta-analyses supporting regulatory or reimbursement decisions was published by D. Jenkins, M. Czachorowski, S. Bujkiewicz et al.[15]. Related to methods and quality of data is the issue of representative sample. E. Gemmen, L. Parmenter and A.B. Mendelsohn look at the most effective approaches to achieve data from real world being representative of the target population[16].

In Brazil there was a research on potential usage of the existing database in health economic analyses with a study focused on acute myocardial infarction[17]. Another use of RWD from databases information can be budget impact calculations[18].

Long-term data collection within a large database can be basis for benefit and patient reported outcomes assessment. Such example is the German registry for psoriasis showing burden of the disease at the time when patient enter into the registry and the quality of care with assessment of the impact on health outcomes[19].

S.J. Rizvi with co-workers indicated that real-world data can confirm the high prevalence of treatment-resistant depression (TRD) and impact the burden of illness associated with TRD in primary care settings in Canada[20].

E. Katodritou with co-workers published their study concluding that RWD are not comparable with the results of the RCTs. However, they may be used to confirm the data of the RCTs and, thus, facilitate the incorporation of certain therapies in standard clinical management[21].

Research in the real world is necessary because of the variety of factors that may play an important role influencing the effectiveness in real life. Factors such as comorbidities, concomitant treatments, adherence, access to care, the strength of the physician-caregiver communication and socio-economic factors among others can modulate the treatment results. Observational studies databases can provide a sufficient level of evidence to support the creation of guidelines i.e., clinical or provide significant information for the decision-making process [22].

Conclusion: Real world data can provide additional information in relation to medical treatments however there is a need to focus attention on methodology of data collection, data quality, potential sources of data and ensure proper legal environment for data collection. Unfortunately, despite large number of publications identified in PubMed database, there are only few available in a form of full text papers, the majority of publications are only abstracts from scientific conferences. In the future in order to share RWD experience it will be of benefit to encourage authors to work on full publications and not to limit their work only to abstracts.■

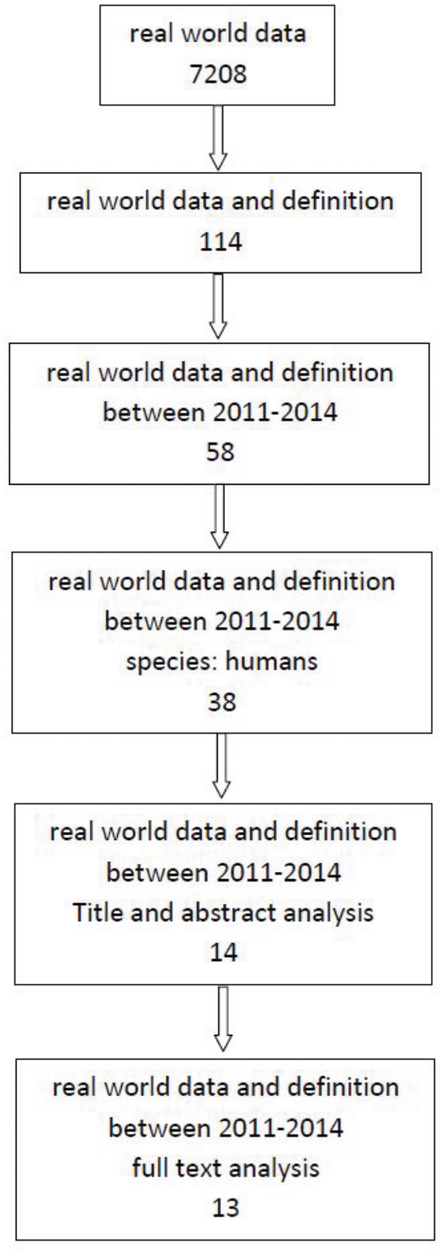

Diagram 1. Search strategies in the library database – PubMed

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#02/2014
ISSN 2299-1247

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