EClinicalMedicine 14 (2019) 11–12

Contents lists available at ScienceDirect

EClinicalMedicine

journal homepage: https://www.journals.elsevier.com/eclinicalmedicine

EClinicalMedicine
Published by THE LANCET

Commentary

A New Tool to Assess the Epidemiology of Immune Thrombocytopenia: The Nordic Country Patient Registry for Romiplostim

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ARTICLE INFO

Article history:
Received 16 August 2019
Accepted 19 August 2019
Available online 29 August 2019

Immune thrombocytopenia (ITP) is a rare autoimmune disease leading to spontaneous bleeding [1]. The knowledge about ITP epidemiology has improved a lot this last decade thanks to the development of clinical registries and the use of electronic health databases [2]. Both types of cohorts have advantages and disadvantages. Therefore, they are complementary sources of data. Indeed, clinical cohorts, even if they are often retrospective due to the rarity of the disease, offer diagnosis ascertainment and the possibility to record detailed behavioral, clinical, laboratory and treatment exposure data. However, they are often limited regarding the number of patients included and missing data is a major concern. In contrast, the use of electronic health databases offers wider populations of patients (sometimes nationwide), the record of data independently from the study, and linkage with some socio-economic, hospital, prescription or dispensing drug data. They also allow cost-effectiveness studies. However, clinical and behavioral data are difficult to handle in these databases; only the patients in contact with the considered health system are captured; eventually, because their primary purpose is not research but collecting data for health economics, the identification of patients need to be ascertained. This is a challenge in diseases like ITP with no routine test for positive diagnosis. Usually, validation studies are conducted in samples by medical chart review to estimate the error due to false positive and false negative cases [3,4]. The Grail is the linkage of data from clinical cohorts with data from electronic health databases, which raises major ethical issues in most countries.

In this issue of *EClinicalMedicine*, Christiansen et al. provide insights on the epidemiology of ITP in adult patients using a new source of data that combines advantages of both clinical cohorts and electronic health database cohorts: the Nordic Country Patient Registry for Romiplostim (NCPRR) [5]. This registry stems from nationwide electronic health records of Denmark, Norway and Sweden. The extent of patient selection, geographically and temporally (2009–2016), overcomes pitfalls of small cohorts. The Scandinavian health system, as well as the recording of hospital specialist outpatient diagnoses, ensure *a priori* an almost completeness of case selection (except patients with asymptomatic and mild thrombocytopenia followed by general practitioners only). Moreover, laboratory result data (notably, platelet counts) are available in the databases, and the retrospective annual review of all medical charts allows the ascertainment of ITP diagnosis as well as the recording of some more detailed clinical data.

The results presented here confirm data regarding the incidence of ITP (overall and by sex and age groups) that were recently demonstrated in other European countries using both clinical registries and electronic health databases [3,6–8]. In that way, this study by Christiansen et al. is an external validation of the NCPRR as a useful tool for ITP epidemiology assessment. New data are also provided regarding the prevalence of chronic ITP (ITP lasting more than 12 months, occurring in about 60% of ITPs in adults) and the prevalence of comorbidities in this population.

Overall, the NCPRR is a powerful tool to assess unsolved questions about ITP epidemiology like variations of incidences, rates of bleeding, thrombosis [9], infections and malignancies. Future pharmacoepidemiological studies are also eagerly awaited to assess in the real-world practice the exposure to ITP treatments as well as their efficacy and safety in the context of lack of comparative clinical trials in this rare disease. More generally, the NCPRR is a striking example of international collaboration for providing quality data on rare diseases.

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DOI of original article: http://dx.doi.org/10.1016/j.eclinm.2019.07.015.
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