Analysis of how research funding is allocated to a wide variety of projects on rare diseases - a case study
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ABSTRACT

Although rare disease research has a high priority in the European Union as well as worldwide, we know very little about how much is actually spent on rare diseases and what topics are really supported. Health research funding comes from three major sources: public sector funding, non-profit funding of the private sector (charities, foundations) and for-profit funding of the private sector and for-profit funding of the private sector. The few studies that investigated the financing of rare disease research reported that disease advocacy groups contribute more to research expenditures, or they defined rare disease as a group of 20 or more diseases. Even less has been published on what research topics got funded and how the magnitude of their support has changed over time. We have performed an analysis through a case study of Rett syndrome, in order to better understand research funding by public and non-profit funding of the private sector in the European Union. We have identified 240 funded projects related to Rett syndrome, the total expenditures on Rett syndrome research was almost 70,000,000 Euros. Although the main research sponsor is the non-profit funding of the private sector, the public sector (European Commission) has significantly contributed to this research field. The peak year of the research funding was 2010. Besides the changes of research expenditure over time, trends in research funding by category for the funded research projects was also described. According to the ORPHANET research categories, while experimental biological studies (mostly gene expression studies) are well supported by both public and private funders, clinical observational studies and sociological health economics studies hardly got funded. Our preliminary results show that the main pillar of this research field is the non-profit funding of the private sector, and experimental research questions attract the majority of research funding.

INTRODUCTION

Although rare disease research has a high priority in the European Union as well as worldwide, we know very little on how much is actually spent on rare diseases and what topics are really supported. Health research funding comes from three major sources: public sector funding, non-profit funding of the private sector (charities, foundations) and for-profit funding of the private sector (pharmaceutical companies). The few studies that investigated the financing of rare disease research reported that disease advocacy groups contribute more to research expenditures, or they defined rare disease as a group of 20 or more diseases. Even less has been published on what research topics got funded and how the magnitude of their support has changed over time. We have performed an analysis through a case study of Rett syndrome, in order to better understand research funding by public and non-profit funding of the private sector in the European Union. We have identified 240 funded projects related to Rett syndrome, the total expenditures on Rett syndrome research was almost 70,000,000 Euros. Although the main research sponsor is the non-profit funding of the private sector, the public sector (European Commission) has significantly contributed to this research field. The peak year of the research funding was 2010. Besides the changes of research expenditure over time, trends in research funding by category for the funded research projects was also described. According to the ORPHANET research categories, while experimental biological studies (mostly gene expression studies) are well supported by both public and private funders, clinical observational studies and sociological health economics studies hardly got funded. Our preliminary results show that the main pillar of this research field is the non-profit funding of the private sector, and experimental research questions attract the majority of research funding.

AIMS AND OBJECTIVES

- To show the magnitude of financial support by public and private organizations and how it has changed over time, and
- To map what research topics got funded. We have performed a case study in order to address these questions, in which we identified and organized relevant research projects carried out in the member states of the European Union during the last 20 years.

METHODS

To obtain data on research expenditures, we applied two approaches. First, official websites of public organizations (European Commission and national research funding) were systematically checked for projects dedicated to Rett Syndrome. Community Research and Development Information Service (CORDIS), 1994; Science Europe (2012). Second, peer reviewed publications on Rett syndrome were identified in the Web of Science, and searched for funding information provided by the authors. The following algorithm was used to select relevant articles. First, the Web of Science was searched with the terms Topic+Rett syndrome (OR Topic+research) for publications. The resulting articles were then searched for information about their funding sources. Websites of these private and public funding organizations were located and searched for research projects on Rett syndrome by using the search terms “Rett syndrome” OR “research”.

The following information has been collected for each research project: administrative number/list/number used to identify the project, project title, project abstract, budget, expenditure of the project, amount of the money, main-executing country, name and type of the funding organization. In order to better understand what research topics get funded, we assessed the research projects by title and abstract and categorized them into groups. Classification of research in this work was based on the classification system research goals according to the ORPHANET: animal model creation, drug development, basic molecular research, clinical trials, drug development, drug delivery, pre-clinical gene therapy, pre-clinical vaccine development, public health/health services study. These groups were then linked to the ORPHANET research categories. The following categories were considered basic research: animal model creation/study, biomarker development, gene expression profile, gene expression/sequence, genetic research, human epidemiology study, in vitro functional study, medical device/instrument development, preclinical trial, pharmaceutical development, post-marketing studies. Among the clinical research, the following categories were considered clinical research: drug development, drug development, drug delivery, pre-clinical gene therapy, pre-clinical vaccine development, public health/health services study. In order to answer our first research question, research funding by funders over time has been mapped. Figure 2 provides an overview of the trends for Rett syndrome research for non-profit private funding, EU and national funding. The figure shows that while the private funders provide support since 1999, public funding became available later, starting from 2004. Research expenditures had two peaks in 2005 and in 2010. Private funding organizations and the EU funding sources are substantially more spending than generous funders: the latter contributes less than 1% of the total funding in recent years. In conclusion, we can state that public funded organzations have a crucial role in ensuring the continuity of research funding on Rett syndrome. However, private organizations also have to be more transparent and have to provide much more information on their spending.

REFERENCES

4. Liu, X. -H., Liu, N., Rice, T. and Liu E. (1999). “Analysis of research topics showed that basic (genetics) studies dominate the research field, other studies hardly got funded.” We recognized certain limitations of the approach taken in our work: 1) the lack of English translation of websites was a major obstacle in finding relevant research projects, and 2) websites often do not report sufficient information on what and to what extent they funded. The lack of available information may also be a serious obstacle for those who seek to access funding possibilities. In conclusion, we can state that public funded organizations have a crucial role in ensuring the continuity of research funding on Rett syndrome. However, private organizations also have to be more transparent and have to provide much more information on their spending.