Evaluating the German national plan for rare diseases

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Objective
National plans (NP) are the main strategic instrument to address the complex issues of rare diseases (RD). In consequence, a concerted NP, the German national plan for rare diseases, was designed to improve health and well-being of people with RD. It consists of a total of 52 proposed actions and associated projects. To support its implementation and to evaluate the effects of the NP an evaluation is being carried out.

Methods
To develop an evaluation concept, a systematic literature review and a comprehensive analysis of the NP and related information was conducted and additionally, national and international experts were interviewed.

Considering the requirements of evaluating complex interventions, a mixed-method approach was developed to assess the impact of the German NP. For each proposed action and all associated projects a web-based questionnaire was used to collect relevant key information from participating stakeholders, followed by a telephone interview to complement the written information with more qualitative insights. This allows a basic assessment of resources allocated to implementation of the action plan, activities, and outcomes. In addition, it provides an opportunity to assess progress and needs for further actions.

The initial phase of the evaluation was carried out from December 2015 to March 2016. As most of the proposed actions and associated projects are managed by more than one responsible person or institution, 99 online-questionnaires were sent out. In total, 69 completed questionnaires could be collected (response-rate: 70%). Additionally, 15 interviews were conducted, covering 39 proposed actions and associated projects.

Results
First analyses show that the implementation process of the German NP is in its early stages. Most of the 52 proposed actions and associated projects are currently being implemented but not yet finalized. Up to now, 8 proposed actions have been finalized, while 7 actions have not yet started (see figure 1).

Less than half of the interviewees stated that they implement their proposed action even without the framework of the NP. However, the same number of actions would not be executed without the framework and some actions would be implemented in a different form, which can be seen as an important success of the German NP (see figure 2).

The implementation of most of the actions is on time. No finalized action was behind schedule. Referring to the ongoing actions, only a minority is behind the scheduled implementation, while 50% of scheduled actions are right on time (see figure 3). Also most actions implemented their contents as planned, while just 18% have to catch up (see figure 4).

The answers of the online-questionnaire were difficult to interpret as just below 50% of the interviewees stated that the proposed actions have specific goals, but most respondents were aware of the content and aims. Furthermore, 70% of actions have no defined duration and just 23% have an associated budget. Therefore interviews were necessary to put previous results in context.

These interviews revealed that the principle of the unanimous vote which is implemented in the NP is the only way to involve all important stakeholders. However, this procedure seems to be somewhat laborious and inefficient for development beyond the conception phase. Particularly as most actions are not clearly defined and demonstrate intent rather than a concrete plan of action. In general, the NP is seen as a good instrument to improve the life situation of people with RD but there is a lack of sustainable financing to maintain or improve the developed structures.

Conclusion
The first phase of the evaluation of the German NP indicates that most of the proposed actions and associated projects are currently underway, but only a few have been implemented as projects with a concrete timeframe and budget. Most proposed actions however have no dedicated budgets. Thus, these actions are mainly driven by the personal motivation of the people involved.

To evaluate NP, a multidimensional concept is needed. Due to the complexity of the German NP, we suggest a multi-level-analysis using a combination of different quantitative and qualitative methods. Within the German context first results suggest ‘information management’, ‘funding’ as well as ‘living and health care conditions’ as key drivers. Further ongoing research in this areas is needed to evaluate the overall impact of the German NP and to guide implementation.

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