Author’s response to reviews

Title: Integrating patient perspectives in medical decision-making: A qualitative interview study examining potentials within the rare disease information exchange process in practice

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Author’s response to reviews:

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Dear Sir or Madame,

Thank you very much for the helpful reviews and for your time spent on the manuscript, contributing to the quality of the manuscript. Please see the section underneath for the point-by-point response to the comments.

Please consider that we have included all changes to the text within our point by point answer. Erased or changed sections have been marked as such. Therefore, we have attached a word version of the point by point answer as this is probably easier to read as the formatting got lost in the section below due to the standardized formatting style.

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Addressing the Reviewer report of Margaret Holmes-Rovner (Reviewer 1):

Please note: We included the changed sections within the point-by-point answer. Erased or changed sections were crossed. Added sections were underlined.

- "Integrating patient perspectives in medical decision-making: A qualitative interview study examining the potential of shared decision-making in practice within the rare
disease information exchange process” is an interview study of the patient and physician experience of clinical encounters. The purpose is to examine the implementation of the shared decision-making concept in the region of Lower Saxony in Germany. The authors further explored whether efficiency potentials exist for the health care system within the rare disease field. The authors suggest this advances the field of shared decision making, since rare diseases have not had much attention. Patient experience of clinical care and decision making may be different from other chronic diseases.

The design is conceptually appropriate. A strength is using the method of Mayring to do qualitative open-ended interviewing within theoretically established a priori categories. In this case, the choice of categories from Charles et al is helpful to addressing the major issues in the field, while still capturing the voices of the interviewees. An inductive-deductive approach was used using the predefined items. This approach, however, steers between over-defined categories and open-ended questioning. The categories taken from Charles et al are: 1. The "patient-physician relationship," 2. "Participation," 3. "Information exchange," and 4. "Decision-making." These provide a framework for the semi-structured interviews. Since the authors did not derive the categories from the interviews, as might be done in a "grounded" approach, it is important for readers to understand how the conversations were structured. This is not possible without examining the interview guide. The guide should be included as an appendix.

-We included the interview guide within the appendix section as appendix number 2.

- The authors make the distinction between the semi-structured questions, and the analytic approach, saying that they, "evaluated the responses based on a qualitative content analysis in an inductive-deductive approach." This process is not fully described. The authors simply state that "all evolving topics in the evaluation process are assigned to the research items and the evolving subcategories". The results suggest they were successful in capturing patient/caregiver perspectives, as some categories specific to rare diseases are reported, such as travel to many specialists and the "psychosomatic corner" reflecting the often-encountered problem that providers may suggest patient symptoms are imagined rather than real.

-We included a description of the inductive-deductive approach of the analysis as follows (the underlined parts were added to the existing description): "An inductive-deductive approach was used. Predefined items were identified in a deductive first step using Charles et al.’s [6] predominately used definition of SDM. Therefore, the following items were noted: 1. The “patient-physician relationship,” 2. “Participation,” 3. “Information exchange,” and 4. “Decision-making.” Second, subcategories were developed, assigned and revised in a stepwise procedure following Mayring’s [33] inductive category development process. [33].”

- The participant recruitment strategy is not well described. This is another problem of incomplete reporting of the strategy and the underlying patient population from which recruitment came. Patients and family members were recruited by the Freiburg Centre for Rare Diseases at the Department of Dermatology of the University Medical Centre. However, it is not clear if a systematic process was used, beyond the need to include a diversity of diseases. Was any kind of balanced sampling done?
Unlike the recruiting approach embedded within the concept of grounded theory, Mayring does not specify any specific sampling technique. Besides, including different rare diseases we also explicitly searched for male participants as females affected were more likely to participate in the study. However, socio-demographic variables show that females were more likely to be recruited nevertheless. Besides, we also strived for the coverage of a broad age range. This was included within the text as follows:

“Patients and family members were recruited by the Freiburg Centre for Rare Diseases at the Department of Dermatology of the University Medical Centre. As “rare diseases” summarizes many conditions with different appearances, the goal was equal coverage of the following disease areas (n = 11): skeletal dysplasia, neuromuscular disorders, immunodeficiencies, genetic eye disorders, genetic skin disorders, connective tissue disorders, genetic kidney diseases, cystic fibrosis and lung diseases, an inherent disturbance of hematopoiesis, inherent metabolic disturbances, and genetic diseases of the digestive tract. However, the interview results often indicated an overall complex, systematic involvement. We also strive for a balanced recruiting of female and male participants, as well as participants from different age groups. We included at least nine patients with a long path to diagnosis, defined as lasting at least 10 years. The inclusion of relatives was necessary, as many rare diseases affect children, who are ineligible for interview, and participants needed to be at least 18 years old. Alternatively, a close relative was invited to answer the questions.”

- The recruitment process is also not described. Was the contact by mail? Telephone? Was it in clinic visits?

-The underlying data base of the University of Freiburg was the clinical register of their rare diseases center. This was included within the text as follows: “[…] The inclusion of relatives was necessary, as many rare diseases affect children, who are ineligible for interview, and participants needed to be at least 18 years old. Alternatively, a close relative was invited to answer the questions. Potential participants were chosen from the rare disease center’s clinical register and were contacted as part of a clinical visit with visiting patients randomly chosen. Patients did not agree to participation in advance. Further, all participants who signed an informed consent agreement and were assigned to an interviewer remained in the study. […]”

- What was the response rate? If identified patients refused, what was the strategy to select the next person to contact?

-All participants who were once assigned to an interviewer of the Health economic center of Hannover remained within the study. This was included within the text as follows: “The underlying data base of the University of Freiburg was the clinical register of their rare diseases center. This was included within the text as follows: “[…] The inclusion of relatives was necessary, as many rare diseases affect children, who are ineligible for interview, and participants needed to be at least 18 years old. Alternatively, a close relative was invited to answer the questions. Potential participants were chosen from the rare disease center’s clinical register and were contacted as part of a clinical visit with visiting patients randomly chosen. Patients did not agree to participation in advance. Further, all participants who signed an informed consent agreement and were assigned to an interviewer remained in the study. […]”
- What kind of data set was used by the Freiburg group? Was it a research database? Was it a marketing data base? Etc.

The underlying data base of the University of Freiburg was the clinical register of their rare diseases center. This was included within the text as follows: “ […] The inclusion of relatives was necessary, as many rare diseases affect children, who are ineligible for interview, and participants needed to be at least 18 years old. Alternatively, a close relative was invited to answer the questions. Potential participants were chosen from the rare disease center’s clinical register and were contacted as part of a clinical visit with visiting patients randomly chosen. Patients did not agree to participation in advance. Further, all participants who signed an informed consent agreement and were assigned to an interviewer remained in the study. […]”

- Had patients agreed in advance to participation in research? What kinds of bias might this introduce?

Visiting patients were randomly chosen. Patients had not agreed to participation in advance. This was included within the text as follows:

“[…] The underlying data base of the University of Freiburg was the clinical register of their rare diseases center. This was included within the text as follows: “ […] The inclusion of relatives was necessary, as many rare diseases affect children, who are ineligible for interview, and participants needed to be at least 18 years old. Alternatively, a close relative was invited to answer the questions. Potential participants were chosen from the rare disease center’s clinical register and were contacted as part of a clinical visit with visiting patients randomly chosen. Patients did not agree to participation in advance. Further, all participants who signed an informed consent agreement and were assigned to an interviewer remained in the study. […]”

- The comparisons among patients, providers and family members would be stronger if the physicians were those who saw these patients. It is understandable that recruitment might make this prohibitive, but it weakens the comparative nature of the analysis. The provider sample represents a diversity of types of physician practice and other providers. However, the level of experience with patients with rare diseases, and the proportion of patients with rare diseases seen by these providers is not described. Thus, it is difficult to know if these are representative of the types of providers patients might see across the spectrum of their search for care.

- Thank you very much for highlighting this difficulty. The following section was included correspondingly:

“The interviewed physicians are part of the field of action of people affected by rare diseases defined as such by Meuser and Nagel [38]. Physicians as health experts serve to supply information from the operating contexts of those affected, covering the overall spectrum of the providing health care structure [38]. Therefore, physicians were selected in accordance with their profession, including general practitioners, specialists, and clinicians. Moreover, guides in the rare disease field were also questioned. The term “clinicians” in Germany represents medical experts working in hospitals, while “specialists” operate in private practice. Guides differ in their
qualifications and are equally trained to direct patients suffering from rare diseases, but were only included in instances in which a medical background could be determined. The Centre of Quality and Management in Health Care, in the State Medical Chamber of Lower Saxony in Hannover, was responsible for recruiting medical professionals. All physicians were recruited within the geographic region of Lower Saxony, representing both urban and rural areas in Germany. As clinical guides occur less frequently, this entire subgroup was recruited from all regions in Germany. The following criteria were employed to create appropriate subgroups: the residence, such as rural, urban, or metropolitan origin; single versus group practice; and basic, regular, specialist, or maximum medical care. Finally, the hierarchy level within the clinical setting, differentiating between assistant physician, senior doctor and chief physician, was also considered. Finally, this study also considered the hierarchy level within the clinical setting, and differentiated between assistant physician, senior doctor and chief physician was also considered. Therefore, the sample covers the heterogeneous area of healthcare provision relative to the research topic of interest, and thus, covers the field of action defined by Meuser and Nagel [38] regarding physicians as experts on rare diseases in Germany.

Results are robust and useful in expanding knowledge of shared decision making to those with rare diseases. The pattern of individual, informed decision-making by patients, followed by paternalistic approaches shows a level of frequent conflict between expert patients and physicians who worry that patients may not follow their recommendations. The dynamic that emerges is one that lacks candor, and that could potentially be well addressed by shared decision making. Adjudicating what counts as good evidence and viable options in this field is challenging.

One minor problem in the organization of the manuscript is that new results appear in the limitations section. These data belong in results if they are to be reported, along with better description of the analytic approach. If these are ad hoc retrospective analyses rather than a priori planned ones, this should be stated. Otherwise, the authors should leave these data out if they do not contribute to understanding the sample.

Thank you very much for these helpful comments. We thoroughly revised the discussion section. We moved sections with new results into the corresponding parts of the results section. Misplaced data or data which does not present the underlying sample was erased. We rearranged the corresponding sections as follows:

“The underlying interview study revealed that medical decision-making as a part of the patient-physician interaction is particularly relevant in the rare disease field due to a high frequency many of medical contacts and a high dependency on the exchange and physicians’ engagement in general.”

“[…] The acknowledgement of symptoms feeds into the patient-physician relationship, which becomes increasingly important in not only the chronic course of rare diseases, but also the often-described frequent interactions with the healthcare system. Therefore, patients Patients […]

“[…] Finally, interactions are also characterized by the many participants involved., as o Other physicians as well as family members can be added to the standard patient-physician interaction.
The volume of transfers—indicated by the term “transfer marathon”—and the number of family members involved with particularly young children suffering from genetic diseases. They illustrates this linked network’s extent. […]”

“ […] This is especially the case in times of diagnosis, as patients search for applicable therapy options and the initiation of medical treatment. Patients also expect physicians to further research these diseases to gather knowledge, although further disappointment occurs when physicians spend too much time to extensively research patient histories. In the patient’s case, the two parties must be separated. Further, the people People affected—either those who have lived with their diseases for a long time, and/or those who have chosen to settle with their diseases and take a more passive role—hand over their responsibilities to the doctor in a preferable “supervisory relationship.” […]”

“ […] In contrast, reports also describe an active, engaged attitude within the healthcare system, in that patients may travel several hundred kilometers to find the right physician or specialized rare diseases center. The subsequent efforts are extensive especially, as young children may suffer from genetic diseases that are also often severe are affected. […]”

“These interviews augment this concept and depict physicians’ difficult role, which shifts from a health information monopoly [51] to a new role as the sorter and structurer of available rare disease information. This role approximates that of a guide, and must be thoroughly differentiated from rare disease guides who operate at a meso-level in Germany, as the communicating of too much information can trigger fear. Literature presents […] “

“ […] Alternatively, a thorough balance is necessary, as too little health information may hinder the effective self-management of those affected. Specifically, the p Potentials exists for patients towho enthusiastically collect data, either from the Internet or from many interactions with experts during their path through the healthcare system. However, this potential is still hindered at a communication level, as the people affected may be concerned with negative reactions in their interactions with professionals. Literature also often describes Internet-based health information searches. For example, McMullan [53] notes different access timeframes, and highlights the threat resulting from information sources of diverging quality [50, 54, 55]. […]”

“ […] Paternalistic approaches were depicted as unavoidable—such as for example in emergency situations—and were also welcomed in cases that threatened responsibility for the health and well-being of children, as the disease burden is then too high for parents to carry. These findings have also been reported by Budych, Helms, and Schulz [56], although these authors do not provide a context in which these approaches were welcome. Baron, Reyher, and Stack [57] report positive outcomes from paternalistic approaches in a crisis situation. The patients in their study were treated paternalistically, and exhibited a higher responsiveness to suggestibility (p > .001), felt they could depend more on the physician, perceived him as warmer and more supportive (p > .01), and expressed fewer incidences of physiological distress compared with patients treated in an egalitarian manner. […]”

“ […] Informed decision-making was also often described to highlight patients’ independence, and especially as they decide when to consult a healthcare professional and the extent to which
patients can take over basic daily care consult their physician. Patients then decide whether to follow physicians’ decisions or change medical consultants. However, this concept was also observed as bearing the highest conflict potential, as it is often associated with questioning the physician’s expertise, possibly leading to controversies between the patient and physician. Thus, it is often assumed that patients prefer Internet-based health information, while concerns simultaneously exist that this would lead to extensive, costly healthcare [52].[…]

- Clearly, the dynamics of a long care-seeking process for patients drives much of their perspectives as reported in the study. The report is appropriately focused largely on patient perspectives. This is a major strength of the study. Perspectives of physicians and family members provide the clinical context and illuminate the constraints and possibilities for expanded shared decision making in this important field. The report is an important first step into further exploration of how information exchange could be improved to support better decision-making across multiple provider settings.

Addressing the Reviewer report of Reviewer 2, requested revisions:

Please note: We included the changed sections within the point-by-point answer. Erased or changed wordings were crossed. Added sections were underlined.

- The aims of the study are not clearly articulated.

-We clarified the aims of the study as follows:

“Triggering this information through effective information exchange strategies, such as shared decision-making, could contribute to the efficiency of, and overall satisfaction with, healthcare systems. In this regard, we first increase the actual usage and acceptance of the concept, than strive to identify the SDM potentials in a rare disease context. In this regard, the purpose of the underlying study is to examine the implementation of the SDM concept in Germany. Further it is explored whether efficiency potentials exist for the health care system within the rare disease field. […]”

- Many aspects of the methods are not clear. For example, the sentence about choosing 3 comparison groups (pg 5, line 23); …

-The section was clarified as follows:

“This study’s empirical evidence is derived from three data sets, offering information on three comparison groups. Three comparison groups were chosen as data sets.”

- … considering the hierarchy level (pg 6, line 20); …

-The underlined section was added for clarification: “Finally, the hierarchy level within the clinical setting, differentiating between assistant physician, senior doctor and chief physician, was also considered.”
- avoiding overestimating SDM effects (pg 6, line 44);

-The underlined section was added as to clarify the sentence: “Thus, we strived to avoid overestimating SDM effects, which can occur due to expressive reporting, when directly inquiring about a concept.”

- approving quotations by a native speaker (p 7, line 32)

-All quotations were approved reread and reviewed by a native speaker.

- Please provide more detail about the interview guide (and the actual guide), including how it was developed.

-The interview guide was included within the appendix section.

- How do the authors know that piloting the guide with 1 patient and 1 family member was enough?

-The following sentences were added as to clarify the issue: “The interview guide was pretested with one patient and one family member, and was then adapted to include the perspectives of participants who have experienced their diseases since birth. The following interviews appropriately covered the different courses of rare diseases, and therefore, this study could optimally cover the different paths through the healthcare system and interactions with healthcare specialists could be ideally covered. The physician’s interview guide was accordingly developed to align the guide’s structure, and was pretested by interviewing one physician (female, 43 years). Piloting the interview guide demonstrated that relevant cases could be appropriately triggered based on the interview guide. Nevertheless, some adaptations were necessary, as the healthcare professionals offered different perspectives on the topic. The research group mutually reviewed both interview guides to align standardized procedures.”

- How many physicians were involved in the piloting of the physician interview guide?

-One. This was included as follows: “The physician’s interview guide was accordingly developed to align the guides’ structure, and pretested interviewing one physician (female, 43 years).”

- More details are needed about the standardized transcription booklet, and the process used for triangulation.

-Regarding the standardized transcription booklet more details were rendered as follows: “All participant interviews were audio recorded, and later transcribed with the aid of F4 transcription software (Version 6, Dr. Dresing & Pehl GmbH in Germany). A standardized interview protocol was also distributed to interviewers of patients and family members to document any special circumstances potentially relevant in interpreting the collected data. A standardized transcription booklet was developed for patients, relatives, and physicians, and was used as a transcription guideline. The transcription booklet offers a standardized definition of different transcription
strategies and codes, where diverging options were possible. This defined the anonymization of participants and locations, as well as the handling of any incomprehensible audio sections.

-The triangulation process was described in more detail as follows: “In the end, Finally, the patients’, family members’, and physicians’ results were triangulated. Evolving items from physicians and relatives’ interviews were matched with already identified items from patients’ interviews where appropriate. Otherwise, a new subcategory was deemed to be necessary.”

-It's very unusual for the term 'inter-subjective reliability' to be used in qualitative research. Can the authors please consider whether this is appropriate and if so, elaborate about its use.

-The wording was changed in accordance with the wording used by Mayring from “inter-subjective reliability” to “formative and summative reliability” as follows: “Finally, two further researchers revised the evolving items to ensure formative and summative reliability;”

-Without more clarity in some of the methods, it is difficult to assess the trustworthiness of the results.

-Please specify the according sections, if any further changes are deemed to be necessary.

-The paper is difficult to follow in many places and could it be written more concisely in some sections (such as the history of shared decision making in the background).

-The history of shared decision making within the background section has been shortened. We have erased the crossed parts as follows:

“The concept of shared decision-making (SDM) was first mentioned as such in 1982 [5]. It has been positioned as a centerpiece between paternalistic models, in which physicians dominate the decision-making process, and an informed patient choice model, in which the physician provides information but the patient assumes a leading role [4, 6]. Various authors have summarized and differentiated these concepts from those in other models [7, 8]; Lin et al. provide an overview of these different definitions [9]. The most often-cited concept originates from the work of Charles et al. [6], who defined SDM as a collaborative process between patient and provider based on a discussion of options, evidence, and potential benefits and harms; this especially considers the patient’s preferences and situations [6]. In this regard, the most prominent aspects of SDM have been identified as being “patient values/preferences” and “options” [10].

A review of literature published between 1996 and 2011 by Blanc et al. [11] identified 1,285 out of 229,179 publications in 15 journals addressing the topic of SDM. In this context, it was identified that publications in medical journals increased exponentially during this period, which indicates the topic’s growing relevance. However, the meaning of SDM is often assumed rather than interpreted through SDM testing models [12]. Other studies suggest that existing SDM models only partially reflect the factors that influence patient empowerment or the breadth of their further potential [13, 14]. Literature focuses on the different indications in evaluating SDM,
and especially in testing the applicability of different realization possibilities, such as decision boxes [15] and online training systems [, 16], or both guidance and caching [, 17].”

- The background section is also quite disjointed and some sentences are not clear (e.g. pg 4, lines 8-10; pg 4, lines 54-60).

- Page 4 lines 8-10: We added the underlined part as to clarify the sentence: “An appropriate level of SDM does occur in practice, although only in approximately 10% of cases [22], suggesting that although literature covers a broad spectrum of the SDM process, further efforts are still necessary to expand its usage.”

-Pg. 4 lines 54-60: We clarified this section as part of the clarification of the aims of the study as follows: “Triggering this information through effective information exchange strategies, such as shared decision-making, could contribute to the efficiency of, and overall satisfaction with, healthcare systems. In this regard, we first increase the actual usage and acceptance of the concept, than strive to identify the SDM potentials in a rare disease context. Therefore, the purpose of the underlying study is to examine the implementation of the SDM concept in Germany. Further, the study explores whether efficiency potentials exist for the healthcare system within the rare disease field. […]”

- I also suggest that the paper is edited with the assistance of a native English speaker. There are many unfamiliar terms and/or terms used in an unconventional manner - for example, p8 , line 35 "stationary setting"

-The paper was reviewed by a professional editing service and re-reviewed considering your comment. All adaptations were included in the track changes modus. The term “stationary setting” was changed into “outpatient care” as follows: “This, and the lack of a constant contact person, and especially within in outpatient care stationary setting, led to high dissatisfaction and patient mistrust.”

Yours sincerely,

Ana Babac