Cardiology in the Young (2010), **20(Suppl. 3)**, 143–148
doi:10.1017/S1047951110001216

Original Article

Patient-reported outcomes in congenital cardiac disease: are they as good as you think they are?

Philip Moons[1,2]

[1]*Center for Health Services and Nursing Research, Katholieke Universiteit Leuven;* [2]*Division of Congenital and Structural Cardiology, University Hospitals Leuven, Belgium*

Abstract Patient-reported outcomes are "any outcome based on data provided by patients or patient proxy as opposed to data provided from other sources". Examples of patient-reported outcomes are quality of life, well-being, functional status, symptoms, adherence to treatment, satisfaction with treatment, and utility or preference-based measures. The main question of this manuscript is whether patient-reported outcomes in patients with congenital cardiac disease are as good as we think they are. In general, we could say yes, because numerous studies show that patients with congenital cardiac disease have an excellent quality of life. By contrast, we could say no, because patients generally overestimate their functioning, and up to two out of three patients are not compliant with the prescribed therapy or recommendations for follow-up. However, most importantly, we have to say that we do not know whether the patient-reported outcomes are good, because research with patient-reported outcomes in congenital cardiac disease is limited. Hence, patient-reported outcomes should be a priority on the agenda for research in the domain of congenital cardiac disease.

Keywords: Congenital cardiac disease; nursing; outcomes; quality of life

Congenital cardiac disease used to be a lethal condition. Indeed, a few decades ago, the life expectancy of patients born with congenital cardiac disease was rather limited. However, owing to improved strategies of surgery, medicine, and intensive care, about 90% of afflicted patients can survive into adulthood to date.[1] Hence, although the main focus of clinicians and researchers used to be a reduction of mortality, this focus has shifted towards issues beyond the quantity of life.[2] Indeed, quality of life has become an important measure of outcome in patients with congenital cardiac disease. Owing to this increasing emphasis on quality of life, an increasing number of studies on the quality of life in children, adolescents, and adults with congenital cardiac disease have been published over the past decades.[3,4]

Correspondence to: P. Moons, Center for Health Services and Nursing Research, Katholieke Universiteit Leuven, Kapucijnenvoer 35, P.B. 7001, B-3000 Leuven, Belgium. Tel: +32-16-336984; Fax: +32-16-336970; E-mail: Philip.Moons@med.kuleuven.be

Quality of life is, however, an equivocal concept. Indeed, no consensus exists on the definition or measurement of quality of life.[5] The term quality of life is often used as a generic label to describe an assortment of physical and psychological variables, which expresses the perspective of the patient. Hence, quality of life seems to be an "umbrella term", covering a potpourri of concepts. This lack of a precise definition of quality of life has contributed to conceptual vagueness and obfuscation.[5] Recognising this problem, Wolfensberger, in 1994, proposed "Let's hang up 'quality of life' as a hopeless term", since it lacks clarity and therefore also utility.[6]

Owing to the quality of life remaining an ambiguous concept, a new construct was created to more accurately and broadly express the perspective of the patients. This new construct is called "patient-reported outcomes".[7] Patient-reported outcomes are "any outcome based on data provided by patients or patient proxy as opposed to data provided from other sources".[7] Together with clinician-reported outcomes,

physiological outcomes, and caregiver-reported outcomes, patient-reported outcomes are an important source of data for the assessment of the outcomes of patients.[7] Patient-reported outcomes are considered

- to provide a unique indicator of the impact of a medical condition,
- to be essential for evaluating efficacy of treatment,
- to be useful for interpreting clinical outcomes, and
- to be a key element in making decisions about treatment.[7]

Examples of patient-reported outcomes are

- quality of life,
- well-being,
- functional status,
- symptoms,
- adherence to treatment,
- satisfaction with treatment, and
- utility or preference-based measures.[7]

Since the early 2000s, interest in patient-reported outcomes has increased. This increased interest is reflected in an accumulating number of articles in the biomedical literature referring to patient-reported outcomes. A search of articles in the database of Pubmed published from 1966 to 2009 identified 1011 articles containing "patient-reported outcome" or "patient-reported outcomes" as a Medical Subject Heading or as a title or abstract term. The number of publications on this subject has grown exponentially (Fig 1). The aim of this paper is to briefly describe what is known about patient-reported outcomes in patients with congenital cardiac disease.

Patient-reported outcomes in congenital cardiac disease

To identify articles that have been published about patient-reported outcomes in congenital cardiac disease, we replicated the search of articles in the database of Pubmed as described above, but added "congenital heart" as an additional search term. This search resulted in no hits. However, a lot of research has already been done on aspects of patient-reported outcomes in congenital cardiac disease, for example, on the quality of life.

Quality of life

Over the past decade, two reviews of the literature on quality of life in patients with congenital cardiac disease have been published.[3,4] The first review was published in 2004 and focused on the conceptual and methodological rigour of studies about quality of life in children, adolescents, and adults with congenital cardiac disease.[3] Overall, 70 articles were reviewed that included 8206 patients. This review revealed the following information:

- 24% of the articles reviewed did not measure quality of life, but drew conclusions in terms of quality of life;
- 43% of the articles did not describe quality of life in the sections of the manuscript about methods or results, but merely in the abstract or discussion;
- 1% of the articles provided a conceptual definition of quality of life;

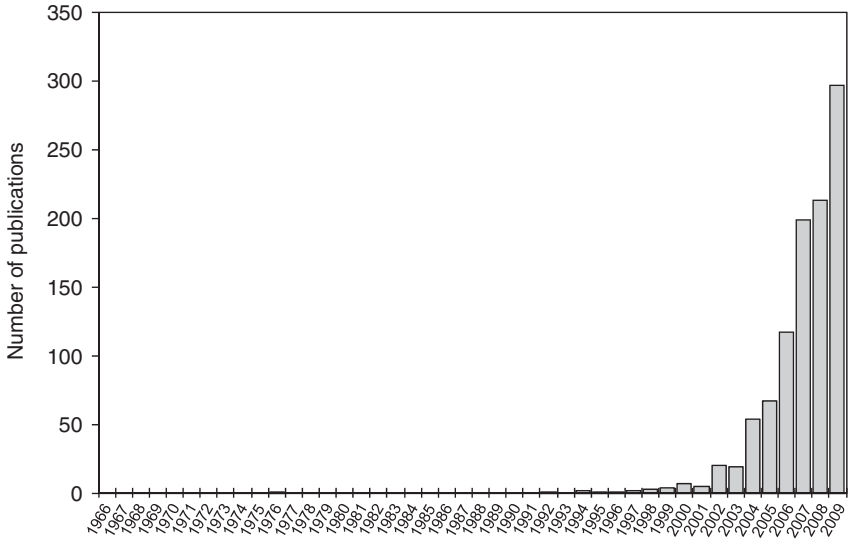

Figure 1.
Number of publications in the database of Pubmed from 1966 to 2009 referring to "patient-reported outcome" or "patient-reported outcomes". (This search was performed on 8 February, 2010.)

https://doi.org/10.1017/S1047951110001216 Published online by Cambridge University Press

- 24% explicitly stated the domains measured as components of quality of life; and
- 3% gave a reason for choosing the instruments used.[3]

The authors concluded that the poor conceptual and methodological basis used in these studies implies that many results of studies about quality of life in patients with congenital cardiac disease were inconclusive. The authors, therefore, plead for more conceptual and methodological rigour with respect to future studies about quality of life.[3]

The second review was published in 2009 and addressed psychological adjustment and quality of life in children and adolescents following open cardiac surgery for congenital cardiac disease.[4] Latal et al identified 12 articles on quality of life in patients aged 2–17 years.

- In four studies, quality of life was comparable to normative samples;
- In four other studies, an impaired quality of life was observed;
- One study found a normal quality of life in 90% of patients;
- One study revealed that patients who underwent arterial switch operation for transposition of the great arteries had a better quality of life than patients after atrial switch repair;
- One study found a low agreement in quality of life assessment between parents and patients; and
- One study addressed the psychometric properties of a quality-of-life instrument.[4]

Direct comparison between the studies is, however, not possible.

Some studies compared the quality of life of adult-aged patients with that of the general population or with normative data.[8–22] In general, previous studies found that quality of life in adult-aged patients is equivalent to that of the general population. When quality of life is measured in terms of functional status, scores of groups of patients are found to be lower than those of normative groups.[15–18,20,22] Conversely, one study found that patients with mild cardiac defects showed a better quality of life, if they did not present with social restrictions.[8] Another study, in which quality of life was operationalised in terms of satisfaction with life, found that adults with congenital cardiac disease had a better quality of life than did their healthy counterparts.[2] In addition, descriptive studies reported an excellent quality of life in patients with congenital cardiac disease.[23]

Taken together, it can be concluded that patients with congenital cardiac disease reported a good quality of life. However, subjective perceptions of patients do not always correspond with objective measurements. A recent study, for instance, found that most patients severely overestimate their physical functioning compared to actual results of exercise testing.[24] Therefore, exercise tests and quality-of-life instruments should be used on a complementary basis,[25] and cannot be used interchangeably.

There exist two major approaches for measuring quality of life:

- the "need approach" and
- the "want approach".[26]

According to the need approach, quality of life depends on fulfilment of basic needs, such as

- good health,
- sufficient mobility,
- good physical performance,
- adequate nutrition, and
- favourable shelter.

In this approach, quality of life is measured using standardised and pre-defined questionnaires about components or determinants of quality of life. This approach assumes that the relative importance of all items is equal among all respondents. The majority of studies about quality of life in patients with congenital cardiac disease use this approach.

By contrast, the want approach assumes that quality of life can only be affected by factors important to an individual.[26] For example, according to the want approach, quality of life depends on

- lifestyle,
- previous experiences,
- ambitions, and
- dreams.[26]

Hence, in this approach, quality of life must be measured with instruments that permit respondents to indicate and, respectively, rate domains that are specifically important for *their* quality of life, that is, individual quality of life. In the field of congenital cardiac disease, studies about quality of life using the want approach are very scant.[27]

The term *quality of life* is often erroneously interchanged with "*health status*" or "*functional status*",[28] and therefore we do not describe these issues separately as distinct elements of patient-reported outcomes in this paper.

Symptoms

Dedicated research about symptoms in congenital cardiac disease is limited. Despite numerous studies anecdotally reporting the prevalence of symptoms in selected groups of patients, to the best of our knowledge, only one study has specifically addressed the experience of symptoms in adults with congenital cardiac disease.[29] This study was a secondary analysis

https://doi.org/10.1017/S1047951110001216 Published online by Cambridge University Press

of data that was collected in a large-scale study about quality of life that included 629 adult-aged patients. The version of the CHD-TAAQOL[30] containing 77 items was used to assess the disease-specific determinants of quality of life. This questionnaire includes 13 items referring to cardiac symptoms. For each item, both the perceived frequency and associated distress were scored. Adults with congenital cardiac disease, including both men and women, reported the following five most frequently occurring symptoms:

- shortness of breath after strolling 1–5 kilometres,
- excessive perspiration,
- dizziness,
- palpitations, and
- getting up often at night to go to the toilet.

Men with congenital cardiac disease reported the following most distressing symptoms:

- difficulty in breathing while lying down,
- shortness of breath after strolling less than 100 metres,
- excess fluid in ankles, legs, and/or abdomen,
- palpitation, and
- shortness of breath after strolling 1–5 kilometres.

Women with congenital cardiac disease reported the following most distressing symptoms:

- fainting,
- difficulty breathing while lying down,
- excessive perspiration,
- severe bleeding due to anticoagulants, and
- palpitations.

Women reported significantly greater frequency of symptoms and greater distress associated with symptoms than men.[29] In conclusion, this study showed that the most frequently occurring symptoms are not necessarily the most distressing ones, and that women with congenital cardiac disease report a higher frequency of symptoms and greater distress associated with symptoms than men with congenital cardiac disease.

Adherence to treatment

The World Health Organization has defined adherence as the extent to which the behaviour of a patient corresponds with the agreed recommendations from a provider of health care.[31] In 2009, a systematic review of the literature with respect to issues of adherence to treatment among children with congenital and acquired cardiac disease, including recipients of cardiac transplants, was published.[32] Only a few studies were identified. In these studies, rates of adherence ranged from 96% for an in-patient programme of exercise, to 33% among those who made all of their medical appointments.[32]

More recently, a study on adherence to oral therapy for anticoagulation after replacement of a cardiac valve with a mechanical prosthesis in 57 patients was published; 25 of these patients had congenital defects of the cardiac valve.[33] This study reported the following findings:

- 72.2% of patients indicated that they were 100% adherent,
- 75% stated that they did not miss one dose of their medication during the last month,
- 14.3% missed one dose during the last month,
- 7.1% missed one dose during the last 14 days,
- 3.6% missed one dose during the last week, and
- no patients reported a drug holiday, which was defined as missing two consecutive doses.[33]

Satisfaction with treatment

As far as we know, two studies have been published on the experiences of patients with the care that they received for congenital cardiac disease.[34,35] Both studies investigated experiences with respect to the transfer from paediatric cardiology towards services for adults. The first study was conducted in a large programme for patients with congenital cardiac disease in the United Kingdom. In this study, 38 patients aged 17–20 years completed a semi-structured questionnaire including open-ended questions. This yielded both quantitative and qualitative data. Patients reported that they more frequently received explanations about treatments and were more involved in the making of decisions in the adult-oriented areas than in paediatric areas. The following issues were important to adolescents and young people in the transition from paediatric cardiology towards services for adults:

- having family/network around,
- being informed,
- being prepared,
- being involved in the making of decisions,
- reassurance of the expertise of specialists,
- gaining confidence in new doctors,
- fitting in with the new team,
- not feeling lost in adult-oriented care, and
- keeping records safe.[34]

In the second study, 14 adolescents with congenital cardiac disease, aged 15–17 years, were interviewed about their expectations and experiences on being transferred from paediatric cardiology to the programme for adults with congenital cardiac disease at a large centre of tertiary care in Belgium.[35] Of the participating patients, four had

https://doi.org/10.1017/S1047951110001216 Published online by Cambridge University Press

been transferred recently and 10 were planned for transfer. Hence, only four patients could report on their actual experiences, whereas the other 10 reflected on their expectations. Adolescents who were already transferred highlighted some key differences between paediatric cardiology and the programme for adults with congenital cardiac disease. For example, these patients were unaware that the two outpatient clinic settings, paediatric and adult, had a different sequence of the stages, such as electrocardiogram, echocardiogram, visit with the nurse-specialist, visit with the cardiologist, and so on. Despite their acknowledging a need to adjust, they also wanted to be informed about these differences. Indeed, adolescents who had had their first visit at the programme for adults with congenital cardiac disease were satisfied with the treatment that they received, but stated in retrospect that they had been largely unaware of what the outpatient visit would entail. Both the adolescents and their parents were unsure of whether parents were allowed to be present during the contacts with the providers of health care.[35]

Utility or preference-based measures

Utility refers to preference-based valuations of the state of health, which are frequently used in evaluations of cost in health care. Measures of utility provide a quantitative estimate of preferences for particular states of health, primarily obtained from a representative sample of the general population. For each state of health, a corresponding index value ranging from 0 to 1 is computed. An index of 0 corresponds to death, while an index of 1 corresponds to perfect health.[36] To date, no utility or preference-based studies in congenital cardiac disease have been reported.

Conclusions

The main question of this paper is whether patient-reported outcomes in patients with congenital cardiac disease are as good as we think they are. In general, we could say yes, because numerous studies show that patients with congenital cardiac disease have an excellent quality of life. By contrast, we could say no, because patients generally overestimate their functioning, and up to two out of three patients are not compliant with the prescribed therapy or recommendations for follow-up. However, most importantly, we have to say that we do not know whether the patient-reported outcomes are good, because

- research with patient-reported outcomes in congenital cardiac disease is limited,
- a strong conceptual framework is not used,
- existing studies have major methodological limitations,
- existing studies cannot be compared, and
- several aspects of patient-reported outcomes are not addressed.

Hence, patient-reported outcomes should be a priority on agenda for research in the domain of the congenital cardiac disease.

References

1. Moons P, Bovijn L, Budts W, Gewillig M. Actual prospects to survive into adulthood in patients with congenital heart disease (abstract). Circulation 2009; 120: S561.
2. Moons P, Van Deyk K, De Bleser L, et al. Quality of life and health status in adults with congenital heart disease: a direct comparison with healthy counterparts. Eur J Cardiovasc Prev Rehabil 2006; 13: 407–413.
3. Moons P, Van Deyk K, Budts W, De Geest S. Caliber of quality-of-life assessments in congenital heart disease: a plea for more conceptual and methodological rigor. Arch Pediatr Adolesc Med 2004; 158: 1062–1069.
4. Latal B, Helfricht S, Fischer JE, Bauersfeld U, Landolt MA. Psychological adjustment and quality of life in children and adolescents following open-heart surgery for congenital heart disease: a systematic review. BMC Pediatr 2009; 9: 6.
5. Moons P, Budts W, De Geest S. Critique on the conceptualisation of quality of life: a review and evaluation of different conceptual approaches. Int J Nurs Stud 2006; 43: 891–901.
6. Wolfensberger W. Let's hang up 'quality of life' as a hopeless term. In: Goode D (ed.). Quality of Life for Persons with Disabilities: International Perspectives and Issues. Brookline Books, Cambridge, MA, 1994, pp 285–321.
7. Acquadro C, Berzon R, Dubois D, et al. Incorporating the patient's perspective into drug development and communication: an ad hoc task force report of the patient-reported outcomes (PRO) Harmonization group meeting at the Food and Drug Administration, February 16, 2001. Value Health 2003; 6: 522–531.
8. Fekkes M, Kamphuis RP, Ottenkamp J, et al. Health-related quality of life in young adults with minor congenital heart disease. Psychol Health 2001; 16: 239–250.
9. Gersony WM, Hayes CJ, Driscoll DJ, et al. Second natural history study of congenital heart defects. Quality of life of patients with aortic stenosis, pulmonary stenosis, or ventricular septal defect. Circulation 1993; 87: I52–I65.
10. Meijboom F, Hess J, Szatmari A, et al. Long-term follow-up (9 to 20 years) after surgical closure of atrial septal defect at a young age. Am J Cardiol 1993; 72: 1431–1434.
11. Meijboom F, Szatmari A, Utens E, et al. Long-term follow-up after surgical closure of ventricular septal defect in infancy and childhood. J Am Coll Cardiol 1994; 24: 1358–1364.
12. Meijboom F, Szatmari A, Deckers JW, et al. Cardiac status and health-related quality of life in the long term after surgical repair of tetralogy of Fallot in infancy and childhood. J Thorac Cardiovasc Surg 1995; 110: 883–891.
13. Meijboom F, Szatmari A, Deckers JW, et al. Long-term follow-up (10 to 17 years) after Mustard repair for transposition of the great arteries. J Thorac Cardiovasc Surg 1996; 111: 1158–1168.
14. Saliba Z, Butera G, Bonnet D, et al. Quality of life and perceived health status in surviving adults with univentricular heart. Heart 2001; 86: 69–73.
15. Kamphuis M, Ottenkamp J, Vliegen HW, et al. Health related quality of life and health status in adult survivors with

previously operated complex congenital heart disease. Heart 2002; 87: 356–362.
16. Lane DA, Lip GY, Millane TA. Quality of life in adults with congenital heart disease. Heart 2002; 88: 71–75.
17. Simko LC, McGinnis KA. Quality of life experienced by adults with congenital heart disease. AACN Clin Issues 2003; 14: 42–53.
18. Simko LC, McGinnis KA. What is the perceived quality of life of adults with congenital heart disease and does it differ by anomaly? J Cardiovasc Nurs 2005; 20: 206–214.
19. Walker WT, Temple IK, Gnanapragasam JP, Goddard JR, Brown EM. Quality of life after repair of tetralogy of Fallot. Cardiol Young 2002; 12: 549–553.
20. Rose M, Kohler K, Kohler F, Sawitzky B, Fliege H, Klapp BF. Determinants of the quality of life of patients with congenital heart disease. Qual Life Res 2005; 14: 35–43.
21. Immer FF, Althaus SM, Berdat PA, Saner H, Carrel TP. Quality of life and specific problems after cardiac surgery in adolescents and adults with congenital heart diseases. Eur J Cardiovasc Prev Rehabil 2005; 12: 138–143.
22. Jefferies JL, Noonan JA, Keller BB, Wilson JF, Griffith C, III. Quality of life and social outcomes in adults with congenital heart disease living in rural areas of Kentucky. Am J Cardiol 2004; 94: 263–266.
23. Moons P, De Bleser L, Budts W, et al. Health status, functional abilities and quality of life following the Mustard or Senning operation. Ann Thorac Surg 2004; 77: 1359–1365.
24. Gratz A, Hess J, Hager A. Self-estimated physical functioning poorly predicts actual exercise capacity in adolescents and adults with congenital heart disease. Eur Heart J 2009; 30: 497–504.
25. Hager A, Hess J. Comparison of health related quality of life with cardiopulmonary exercise testing in adolescents and adults with congenital heart disease. Heart 2005; 91: 517–520.
26. Häyry M. Measuring the quality of life: why, how and what? In: Joyce CRB, O'Boyle CA, McGee H (eds). Individual Quality of Life: Approaches to Conceptualisation and Assessment. Harwood Academic Publishers, Amsterdam, 1999, pp 9–27.
27. Moons P, Van Deyk K, Marquet K, et al. Individual quality of life in adults with congenital heart disease: a paradigm shift. Eur Heart J 2005; 26: 298–307.
28. Moons P. Why call it health-related quality of life when you mean perceived health status? Eur J Cardiovasc Nurs 2004; 3: 275–277.
29. Moons P, Van Deyk K, Marquet K, De Bleser L, Budts W, De Geest S. Symptom experience in adults with congenital heart disease (abstract). Eur Heart J 2006; 27: 791.
30. Kamphuis M, Zwinderman KH, Vogels T, et al. A cardiac-specific health-related quality of life module for young adults with congenital heart disease: development and validation. Qual Life Res 2004; 13: 735–745.
31. Sabate E. Adherence to Long-term Therapies: Evidence for Action. World Health Organization, Geneva, 2003, 1–194.
32. Ittenbach RF, Cassedy AE, Marino BS, Spicer RL, Drotar D. Adherence to treatment among children with cardiac disease. Cardiol Young 2009; 19: 545–551.
33. Van Damme S, Van Deyk K, Budts W, Verhamme P, Moons P. Patient knowledge of and adherence to oral anticoagulation therapy after mechanical heart-valve replacement for congenital or acquired valve defects. Heart Lung 2010 (in press).
34. Wray J, Maynard L. Specialist cardiac services: what do young people want? Cardiol Young 2008; 18: 569–574.
35. Moons P, Pinxten S, Dedroog D, et al. Expectations and experiences of adolescents with congenital heart disease on being transferred from pediatric cardiology to an adult congenital heart disease program. J Adolesc Health 2009; 44: 316–322.
36. Dolan P, Gudex C, Kind P, Williams A. Valuing health states: a comparison of methods. J Health Econ 1996; 15: 209–231.