Review article

Outcome evaluation in pediatric orthopedics

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ABSTRACT

Outcome evaluation is an objective procedure to determine the impact and success of a therapeutic program, focusing on the patient’s well being in daily life. It is important not to confuse the different concepts found in the health status classification and quality of life evaluation. Both are suitable for outcome evaluation and may be used according to the principles of evidence-based medicine. Outcome evaluation of a therapeutic program may be compared to a search in the literature and the level of evidence. The goal is to achieve direct benefits for the patient and society. Strategies to improve daily practice may be developed. Scientific societies can create database for outcome evaluation and develop standardized protocols for longitudinal outcome follow-up, as well as proposing this type of study to health authorities. Based on these results, a global view of the patient can be taken into consideration to influence medical, socioeconomic and health management patient care.

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1. Introduction

In 1981, the World Health Organization (WHO) defined an evaluation as “a systematic way to learn lessons from different experiences...to improve existing activities and promote more effective planning by making a judicious choice among possible future actions. This implies a critical analysis of the different aspects of the development and execution of a program and its activities, its pertinence, its formulation, its effectiveness and efficacy, its cost and its acceptance by all interested parties” (Box 1).

For many years, day-to-day clinical therapeutic decisions were made based on series of uncontrolled cases and expert opinions, or the opinions of eminent senior colleagues, which played an important role in this process [1]. Because all of these opinions were probably not justified, certain patients may not have received the best treatment.

Although the topic of including quality of life evaluations in orthopedic surgery was presented at a teaching conference by Guillemin in 2009 [2], how should we approach outcome evaluation in children? An understanding of the methodological bases of outcome evaluation and knowing how to apply them to daily practice is essential if the quality of treatment programs is to be improved.

Historically, the beginning of outcome evaluation can be found in the work by Semmelweis in 1847 when he identified the association between puerperal fever and a doctor’s failure to wash his hands, a study whose value was only recognized after the author's death when the role of bacteria in infections had been confirmed. This is an early example of an outcome evaluation study, which is characterized by a continuous collection of detailed statistics.

At the beginning of the 20th century, professional organizations and hospital authorities introduced the use of standardized medical files. Standardized data collection meant that medical file database could be used as a fairly reliable source of information for research.

Since the 1960s, the study of “outcome” has become a research method of its own [3]. The term «outcome management» was invented to guide care by performing a detailed analysis of the treatments used for the patient’s disease [4].

In 1972, the study by Archie Cochrane clarified the research concepts of outcome evaluation and evidence-based medicine (EBM) [5].

The experiences and preferences of patients have been more effectively taken into account since the 1990s. At the same time,
the need for information by healthcare payers and organizations, as well as by the public must be respected [6].

There are now many research topics in outcome evaluation including:

- patient safety;
- the efficacy of policy decisions/their implementation by clinicians;
- equal access to healthcare (ethnic origin, gender, socioeconomic status of patients);
- cost saving;
- exploring potential ways to improve the reactivity of the system:
  - ways to improve patient care practices,
  - databases and registers that are often fragmented,
  - results that are reported inappropriately by numerous hospitals,
  - insufficient funds specifically allocated for this purpose,
  - limited number of researchers able to perform high quality studies.

Therapeutic management of our young patients must evolve in relation to the results of this research. We are moving towards therapeutic programs based on scientific evidence that is focused upon the patient’s well being in his/her daily life.

Outcome evaluation is a field of research that is so vast that it is impossible for a surgeon to be familiar with all of its aspects. These are multidisciplinary studies, including healthcare practitioners, medical device and pharmaceutical suppliers, health economists, sociologists, and public health researchers.

If a surgeon understands the logic of this research method, s/he rapidly realizes its usefulness in daily professional practice and in the search to improve treatment programs.

2. Concepts and tools of outcome evaluation

Outcome evaluation is a systematic and objective procedure to determine the success and impact of a treatment program. This evaluation asks the question of “why” this treatment and “how” it is managed and determines to what extent targets and goals have been reached.

To perform an outcome evaluation, a systematic, structured and consistent methodology must be implemented to collect, analyze and use information to answer a clearly stated question.

The six steps are:

- form a team to plan and implement the evaluation;
- effectively plan the evaluation:
  - what is the goal of the evaluation?
  - construct a model for the evaluation,
  - define measurable goals,
  - identify the context of the evaluation,
- determine the steps of the evaluation;
- collect the data for the evaluation: develop procedures and tools for data acquisition;
- analyze evaluation data and obtain feedback throughout the study;
- prepare a report of the evaluation including an interpretation of results to determine the efficacy of the therapeutic program.

What is the relevance of this type of evaluation within the larger scientific context? Indeed, it is important not to confuse the concepts of classification of health status with a quality of life evaluation. They both have a role to play and we define each of them in the next chapter. Finally, evidence-based medicine (EBM) can help improve the quality of service provided to patients.

2.1. World Health Organisation International Classification of Functioning, Disability and Health (ICF or ICH 2)

The ICF is a precious tool to describe and compare health in populations around the world [7]. It is the theoretical model of reference when designing evaluation studies.

It is a polyvalent system of classification developed for use in diverse sectors and disciplines. It applies to all types of health status associated with any type of disease.

The ICF can be used for various purposes:

- as a clinical tool – to evaluate needs, choose the most well adapted treatment, evaluate professional aptitude, evaluate rehabilitation;
- as a statistical tool;
- as a research tool
- as a tool to develop and implement social and health policies
- as a pedagogical tool – to design programs and/or to develop awareness campaigns

When the study is being designed, the ICF domain that is targeted must be identified. Each domain has specific procedures or measurement instruments, with their own psychometric qualities (Box 2) [8,9].

“The functioning and disability of a person caused by a disease is the result of the dynamic interaction between his/her state of health and contextual factors” (Fig. 1). This introduces the concepts of “activity” and “participation” into the context of personal and environmental factors.

The ICF systematically groups together the different domains that a person with a specific health problem will encounter.

Thus, the “functional status” of a patient refers to what an individual is capable of achieving within the context of a social role that s/he has been assigned; it therefore refers to a person’s performance of specific tasks, such as day-to-day activities. On the other hand, when we refer to a “state of health”, we are speaking of medical and functional well being on a larger scale, which can be reported in terms of “the impact of a disability” [10,11].

Thus, the ICF includes “building blocks” (“a medical condition”, “anatomical structure/function”, “activity”, “participation”, “personal factors”, “environment”) that the user can assemble to build his/her own evaluation models.

The evaluation can then be based on different study designs:

- for the treatment of a population: controlled randomized trials, cross-sectional studies, observational cohort studies, a pre- or post-design or controlled prospective studies;
- meta-analyses and systematic reviews based on a study of the literature;
- simulations to predict future effects;
- studies of healthcare facilities, economic assessments, analysis of administrative structures;
- studies to influence public policy, or;
- patient questionnaires that can be adapted.

These concepts have often been misused in the literature. Most studies are limited to the dimension “anatomical structure/organic function”. Recently, studies targeting the patient’s daily life, their activities and their participation in a particular environment have been published, reporting a patient’s actual life.

2.2. Quality of life evaluation

Quality of life is a term that is in vogue and is often misused as an umbrella to cover any type of self-assessment. When using
this conceptual framework, the concept must be defined and placed within a clinical context [11].

Quality of life (QOL) follows its own methodology and is not part of the ICF per se. This can lead to confusion when a patient evaluation is planned. The experts in the field have not reached a consensus on the role of a QOL evaluation. Certain ICF factors in the domains of “environment” and “personal factors” may be included, but do not directly correspond to this principle. Therefore, when designing evaluation studies, it is more suitable to use measurement tools for each ICF domain while also associating specific QOL tools of measurement.

Quality of life explores all aspects of health as defined by WHO: physical, psychological and social and function is not limited to a specific environment as described in the ICF. The three domains are indeed associated and QOL does not merely measure the purely physical dimension.

Thus, the quality of life of a child with a physical disability is not necessarily poor, despite what healthy individuals may imagine [12].

Specialists differentiate QOL and Health-related Quality of Life (HRQOL). QOL is more a term of overall well being [13], including the perceived importance of physical health by the person, where s/he lives and spends his/her time and if s/he has friends and access to education and work.

The HRQOL targets the components of health associated with satisfaction in life, personal hygiene, mobility and communication [14]. Measurement of HRQOL is defined as multidimensional. It explores all of the dimensions of health found in the WHO definition:

- physical (autonomy, physical ability, ability to perform daily tasks, pain...);
- psychological (well being, anxiety, emotivity...);
- social (relationship with family, friends, professional environment...) in relation to a medical condition (state of health) [10,15].

The concepts “activity” (= performing a task; or the fact that an individual does something) and “participation” (= involvement in a real life situation) of the ICF are also multidimensional. Unlike the subjective concept QOL, these are objective concepts that can be evaluated with self-assessment measurement tools. Nevertheless, they do not explore the same domains [16] (Table 1).

An HRQOL questionnaire makes it possible to measure, elaborate and validate the subjective feelings reported by the subject. It is an indicator of the perceived state of health.

The well-informed clinician who wishes to evaluate quality of life should be familiar with the main aspects of the development and validation of the HRQOL scale, so that s/he can choose the most appropriate questionnaire when planning his/her study.

Developing questionnaires is a long process requiring a multidisciplinary team. These are inexpensive non-invasive evaluation tools. These tools are differentiated by the way they are developed, administered, and by their field of application (Table 2).

Although including QOL measurements in the global evaluation of children with orthopedic diseases goes beyond the strict framework of the ICF, in our opinion, it is indispensable to assess all aspects of a patient’s life, and to establish a global, long-term therapeutic program (Table 3).

2.3. Evidence-based medicine

With evidence-based medicine (EBM), clinical decisions are not only based upon theoretical knowledge, judgment and experience which are the main elements of traditional medicine, but also upon scientific “evidence”, while taking into account the patients’ preferences.

By “evidence”, is meant knowledge derived from systematic clinical research, mainly in the domain of the diagnosis and treatment of patients, based on valid results applicable to regular medical practice.

Clinical studies that may be included are randomized controlled studies, meta-analyses, but also well-designed cross-sectional studies or follow-up studies to evaluate a diagnostic test or for the prognosis of disease progression [17].

The concept of EBM was developed by the Canadian epidemiologists at the McMaster Medical School then adopted by the Cochrane Collaboration [18].

This “factual medicine”, which is the French translation of EBM is based on two fundamental principles:

- the clinical decision is based on a rigorous analysis of the patient’s condition. All assertions and knowledge must be evaluated according to their level of evidence;
• the benefits and risks of each diagnostic or therapeutic option should be determined as precisely as possible for each patient.

The steps of EBM have been clearly described [17,19]: asking a pertinent clinical question in relation to a specific health problem, finding the best evidence in the scientific literature, evaluating the evidence in relation to the clinical question, and the level of evidence of the study [20] and finally, including the results in regular clinical practice [21].

The literature is analyzed by classifying the references in the literature according to their level of evidence, that is based on a standardized grade of scientific validity of a published (available) study, in relation to the quality of the methodology and how it was performed, analysis of the results and the pertinence of the conclusions. Once they have been scientifically validated, the results can then be adapted to the practices in the specific medical field, then adopted by practitioners and his/her patients, and finally included in guidelines (Table 4).

Although this seems to be a very concrete approach, observation and evaluation of medical practices in the field shows that this is not so, if only because EBM terminology remains unfamiliar for the orthopedic surgeon.

The integration of EBM is slow because it is considered to be difficult, time-consuming and confusing, there are very few studies in the literature with a high level of evidence to confirm that patients are receiving the most well adapted treatments [1].

For example, does surgical correction of 60° idiopathic scoliosis with significant radiographic improvement improve the patient’s adult quality of life? Or, does arthrodesis improve a patient’s daily life, their ability to participate in a social life and their professional choices? What microeconomic conclusions can be drawn about this treatment?

Although it is not necessary to be an expert in EBM, it is important to know the EBM level of a report and whether it is a high or low quality study. In this way, one becomes an informed consumer of the medical literature, potentially making better decisions for one’s patients, and capable of passing on this culture by teaching it to younger colleagues [22].

Applying the concepts of EBM does not mean that a patient’s individual needs are not taken into account [1]. It just means that the research with the best evidence becomes part of clinical expertise and patient values [17].

Orthopedists are gradually including EBM in their practice, in particular, by associating the concept of level of evidence into scientific journal abstracts [23] and meetings and by offering seminars on this topic to scientific societies or encouraging practitioners to follow specific training courses on this topic.

Thus, the concepts of ICF, QOL evaluations and EBM work perfectly well together. Research evaluating the outcome of prior treatment is an important aid to the EBM approach. The goal is to provide direct benefits to the patient and society as well as to develop strategies to improve clinical practices (Fig. 1).

3. The problem of pediatric orthopedics

In the past 20 years, the field of neuro-orthopedics has reaped the most benefit from the methodological progress made in outcome evaluation (Fig. 2). A systematic approach to the global evaluation of patients with cerebral palsy, which has been promoted by pediatric orthopedists defending the use of evidence-based medicine [1,24], has significantly improved patient management.

However, the approach to outcome evaluation is not the same for all pediatric orthopedic diseases.

Studies focus mainly on the “structure and function” domain of the ICF, for example, by measuring change by clinical examination of joints or radiographic angles. In fact, we know very little about the real impact of pediatric orthopedic treatment on children’s daily life, future function, social and professional integration or its short, medium and long-term medicoeconomic impact. And yet, these factors must be understood if health care authorities are to adapt their financial management and develop social aid strategies.

3.1. Specificity of evaluating outcome in children

Therapeutic outcome evaluation in adults cannot be directly applied to children. The adult is directly concerned by the disabilities caused by his/her disease, which influence his/her possibilities of social integration. A child only realizes his/her difference in relation to others later, and does not understand the permanent nature
Table 1
Motor skills and performance scales (ICF domains “activity” and “participation”).

<table>
<thead>
<tr>
<th>Name of the scale</th>
<th>Type</th>
<th>Use in children</th>
<th>French validation</th>
<th>Subject explored</th>
<th>Behavior</th>
<th>Communication/ Locomotion</th>
<th>Manipulation/ use of body for certain tasks</th>
<th>Physical care</th>
<th>Disabilities revealed in certain situations</th>
</tr>
</thead>
<tbody>
<tr>
<td>CHART (Craig Handicap Reporting &amp; Assessment Technique)</td>
<td>1 or 2</td>
<td>0</td>
<td>0</td>
<td>5/6 survival roles</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>PEDI (Pediatric Evaluation of Disability Inventory)</td>
<td>2 or 3</td>
<td>1</td>
<td>0</td>
<td>Functional abilities</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>London Handicap Scale</td>
<td>3</td>
<td>0</td>
<td>0</td>
<td>Quantification of the handicap</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>FMH (Fertigkeitskala Münster Heidelberg)</td>
<td>2 or 3</td>
<td>1</td>
<td>0</td>
<td>Performance/QOL</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>COPM (Canadian Occupational Performance Measure)</td>
<td>3</td>
<td>1</td>
<td>0</td>
<td>Activity/participation</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>POSNA instrument (Pediatric Outcomes Data Collection Tool)</td>
<td>1 or 2</td>
<td>1</td>
<td>0</td>
<td>Level of function in real life</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>0</td>
</tr>
<tr>
<td>ASK (activities scale for kids)</td>
<td>1</td>
<td>1</td>
<td>0</td>
<td>Physical activity in the environment</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>LAQ-CP (Lifestyle Assessment Questionnaire)</td>
<td>2</td>
<td>1</td>
<td>0</td>
<td>Activity, participation</td>
<td>1</td>
<td>1</td>
<td>1</td>
<td>0</td>
<td>1</td>
</tr>
<tr>
<td>AMPS (Assessment of Motor and Process Skills)</td>
<td>3</td>
<td>1</td>
<td>0</td>
<td>Activity in daily life</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>CAPE (Children’s Assessment of Participation and Enjoyment)</td>
<td>1, 2 or 3</td>
<td>1</td>
<td>0</td>
<td>Participation and amusement</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>HSC (Handicap scale for children)</td>
<td>1</td>
<td>1</td>
<td>0</td>
<td>Social participation</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>M-ADL (Münchener ADL-Fragebogen)</td>
<td>2 or 3</td>
<td>1</td>
<td>0</td>
<td>Activities in daily life</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>PS-ADL (Performance and Satisfactory in Activities of Daily Living)</td>
<td>3</td>
<td>0</td>
<td>0</td>
<td>Daily Activities</td>
<td>0</td>
<td>0</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>LIFE-H (Assessment of Life Habits for Children)</td>
<td>1 or 2</td>
<td>1</td>
<td>1</td>
<td>Activity, participation and QOL scale</td>
<td>1</td>
<td>1</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
</tbody>
</table>


Table 2
Types of quality of life evaluation questionnaires.

<table>
<thead>
<tr>
<th>Type of development</th>
<th>Description</th>
<th>Disease specific: sensitive to clinical variations</th>
<th>By a third party (physician/caretaker)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Type of administration</td>
<td>Self-assessment (patient him/herself)</td>
<td>And/or for each dimension (profile) studied</td>
<td></td>
</tr>
<tr>
<td>Targeted Age group</td>
<td>Small child, child, adolescent, adult</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Types of response to questions (items)</td>
<td>Yes/No Visual analogic scale Increasing intensity mode</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Questionnaire results</td>
<td>Global score (index)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Domains evaluated</td>
<td>Physical activities: capacities to participate on daily life activities</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Psychological status: emotional and mental status of well being, including status of depression, anxiety, concern, guilt, enjoyment, dynamism, optimism</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Social activities, relations with others, capacities, participation in the various activities of the life of relation; Overall satisfaction: overall feeling having (or not) a pleasant life</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Perception of its own health status, positive or negative (depending on age, its temporal references)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Complementary domains: pain, sleep, cognitive status, sexual satisfaction</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
### Table 3
Quality of life questionnaires in children.

<table>
<thead>
<tr>
<th>Instrument</th>
<th>Year</th>
<th>Country/Language</th>
<th>Targeted age</th>
<th>Type of administration</th>
<th>Number of Questions</th>
<th>Domains explored</th>
<th>Time to fill out (min)</th>
<th>PMV*</th>
<th>Validation of French version</th>
</tr>
</thead>
<tbody>
<tr>
<td>Generic QOL scales in children</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Satisfaction, Discomfort, Moral resources, Avoiding risk, Accomplishment</td>
<td>30</td>
<td>TtT</td>
<td>No</td>
</tr>
<tr>
<td>CHIP (Child Health Questionnaire)</td>
<td>1996</td>
<td>US/English French</td>
<td>6–11</td>
<td>SAQ: CHIP-CE</td>
<td>6</td>
<td>Physical, Physical function, Physical pain, Sociophysical role, General perception of health, Mental health, General behavior, Self esteem, Emotional influence of parents, Parental influence over time, Family influence</td>
<td>20</td>
<td>VIS</td>
<td>Yes</td>
</tr>
<tr>
<td>AUQUEI (Auto-Questionnaire de l'Enfant Image)</td>
<td>1997</td>
<td>France</td>
<td>4–12</td>
<td>SAQ</td>
<td>26</td>
<td>Family relationships, Social, Activity (play, school life, leisure time), Health, &quot;Functions&quot; (sleep, eating habits), Separation</td>
<td>NR</td>
<td>VIS</td>
<td>Yes</td>
</tr>
<tr>
<td>GCQ (Generic Children's Quality of Life Measure)</td>
<td>1997</td>
<td>UK</td>
<td>6–16</td>
<td>SAQ</td>
<td>25 × 2 (50)</td>
<td>Physical function (sports), positive emotions, self image, cognitive function, relationships with friends, interaction with family Two step evaluation: &quot;I feel most like myself&quot; &quot;I would most like to be&quot; Physical and emotional well being, Self esteem, Family, Friends, Daily functioning, (school or kindergarten/day care)</td>
<td>NR</td>
<td>VIS</td>
<td>No</td>
</tr>
<tr>
<td>KINDL</td>
<td>1998</td>
<td>Germany</td>
<td>4–7</td>
<td>SAQ: Kiddy</td>
<td>10</td>
<td>Pain and symptoms, Cognitive function, Social function Positive global emotional function Global negative emotional function Physical, emotional, social, scholastic function</td>
<td>10</td>
<td>VIS</td>
<td>No</td>
</tr>
<tr>
<td>TACQOL</td>
<td>1998</td>
<td>Holland</td>
<td>8–15</td>
<td>SAQ</td>
<td>53</td>
<td>Basic motor function autonomy, Self image, relationship with friends, scholastic function, interaction with family Evaluation in two steps: &quot;I like me&quot; &quot;I would like to be&quot;</td>
<td>10 (parents)</td>
<td>VIS</td>
<td>No</td>
</tr>
<tr>
<td>PedSQL (Pediatric Quality of Life Inventory)</td>
<td>1999</td>
<td>US</td>
<td>5–7</td>
<td>SAQ</td>
<td>23</td>
<td>Activity limitations, physical symptoms (headaches), negative feelings, (anxious, trouble sleeping) self image, relationship with friends, scholastic function, interaction with family Evaluation in two steps: &quot;I like me&quot; &quot;I would like to be&quot;</td>
<td>5–10</td>
<td>VIS</td>
<td>No</td>
</tr>
<tr>
<td>EHRQL (Exeter HRQL scale)</td>
<td>1999</td>
<td>UK</td>
<td>6–11</td>
<td>SAQ</td>
<td>16 pictures</td>
<td></td>
<td>20</td>
<td>VIS</td>
<td>No</td>
</tr>
</tbody>
</table>
Table 3 (Continued)

<table>
<thead>
<tr>
<th>Instrument</th>
<th>Year</th>
<th>Country/Language</th>
<th>Type of administration</th>
<th>Targeted age</th>
<th>Number of Questions</th>
<th>Domains explored</th>
<th>Time to fill out (min)</th>
<th>PMV*</th>
<th>Validation of French version</th>
</tr>
</thead>
<tbody>
<tr>
<td>DHP-A (DUKE Health Profile-adolescent version)</td>
<td>2005</td>
<td>UK</td>
<td>SAQ</td>
<td>SAQ: 13–18</td>
<td>17</td>
<td>Health: Physical, Social, General, Perceived, Self Esteem, Anxiety, Depression, Pain, Disability</td>
<td>NR</td>
<td>VIS</td>
<td>TrT</td>
</tr>
<tr>
<td>KIDSCREEN</td>
<td>2005</td>
<td>Europe</td>
<td>SAQ</td>
<td>SAQ: 8–18; PQ: 8–18</td>
<td>52/27/10</td>
<td>Physical well being, Positive and negative psychological effects, Self esteem, Family life, Financial resources, Relationship with friends, School, Social Integration Physical, psychological, social</td>
<td>NR</td>
<td>VES</td>
<td>Vcult</td>
</tr>
<tr>
<td>kidQol</td>
<td>2005</td>
<td>France</td>
<td>SAQ</td>
<td>SAQ: 6–12</td>
<td>44</td>
<td>Friendship and social aspects, General Health, Physical health, Academic achievement, Personal relationships, Peer relationships, Family relationships, Interest in school, Cultural and religious activities, Religious beliefs, Religious participation, Future orientation, Pastime and leisure activities, Physical appearance</td>
<td>NR</td>
<td>NR</td>
<td>Yes</td>
</tr>
</tbody>
</table>

Specific quality of life scales for cerebral palsy or specific neuromotor diseases

<table>
<thead>
<tr>
<th>Instrument</th>
<th>Year</th>
<th>Country/Language</th>
<th>Type of administration</th>
<th>Targeted age</th>
<th>Number of Questions</th>
<th>Domains explored</th>
<th>Time to fill out (min)</th>
<th>PMV*</th>
<th>Validation of French version</th>
</tr>
</thead>
<tbody>
<tr>
<td>DISABKIDS</td>
<td>2006</td>
<td>Europe</td>
<td>SAQ</td>
<td>SAQ: 8–16; PQ: 4–7; PQ: parents from 4–16</td>
<td>37/12 module IMC: 7</td>
<td></td>
<td></td>
<td>NR</td>
<td>VES</td>
</tr>
</tbody>
</table>

SAQ: Self-assessment questionnaire; PQ: Parent/caretaker questionnaire; HRQOL: Health-related quality of life; QOL: Quality of life; NR: not reported in the publications; PMV: Psychometric validation; TrT: Test re-test Reliability; VES: Validity of external structure; VT: Validity of a trait; VClin: Clinical validity; VIS: Validity of internal structure (also validity of design); Vcult: Transcultural validity.

* Adapted according to the references [7–9].
of his/her handicap before puberty. Until then, the child’s family does everything possible to adapt his/her environment to the disability. This makes a child’s perception of his/her disability more difficult because s/he has no notion of what is “normal”. This gradual awareness affects the child’s judgment and one day s/he no longer accepts what may have been acceptable until then.

The ICF did not sufficiently take into account the growth and changes that take place during the first twenty years of life. The International classification of disability and health in children and youth (ICF-CY) [25] has corrected this limitation. Although it confirms the importance of precisely describing the state of health of children based on a methodology that has been shown to be effective in adults, it also places children and young patients within the continuum of their environment and their development. Details and descriptive categories specific to babies, children and adolescents have been added.

With the ICF-CY, public authorities, health and sociomedical professionals, associations of handicapped individuals, parents, healthcare service users and researchers have a tool that specifically describes and measures the important characteristics of growth, health, development, function and disabilities during childhood and adolescence, and also identifies the health, education and social needs of the child. The ICF-CY serves as a framework of reference for public and associational policy on childhood disabilities.

### 3.2. Evaluation of quality of life in the child

The concept of health perception in children is not comparable to that of an adult, and quality of life questionnaires designed specifically for children must be used. At present, there is no consensus on quality of life in children. Most of the tools have been developed from adult tools. The influence of family relationships and the school environment are important [26].

One recent review explored the possibilities for evaluation: are there specific HRQOL tools for children and adolescents? In fact, these are merely generic tools for comparison between healthy and chronically ill children and adolescents [27].

Self-assessment with a self-administered questionnaire is considered to be the best method of evaluation. In young children or in those with cognitive disorders, the questionnaire can either be

---

**Table 4**

Level of scientific proof (adapted from the Sackett score).

<table>
<thead>
<tr>
<th>Level of scientific evidence in the literature</th>
<th>Strength of the recommendation</th>
</tr>
</thead>
</table>
| Level 1
  - Robust randomized comparative studies (appropriate information from at least one randomized study: with enough subjects, and clear methods): meta-analyses | Grade A
  - Based on well established scientific proof from studies with a high level of evidence |
| Level 2
  - Non-randomized controlled studies | Grade B
  - Based on strong scientific proof provided by studies with an intermediate level of evidence |
| Level 3
  - Well performed recent, non-randomized case-control studies (in more than one center, or in more than one research group) | Grade C
  - Based on studies with a low level of evidence |
| Level 4
  - Comparative trials of retrospective series, information obtained from multiple chronological series, with or without an intervention | |
| Level 5
  - Expert opinions, based on clinical experience, descriptive studies (case studies) or reports from expert committees | |

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**Fig. 2.** Association of evaluation tools by ICF domains and quality of life in neuro-orthopedics; patient with bilateral spastic cerebral palsy.
filled out by the child or by someone close to the child (third party evaluation), usually by the child’s family or caretakers. However, in this case the information obtained is obviously different. Studies have shown that responses from different respondents (children and parents, for example) were far from being in perfect agreement [28].

The interaction of other neurodevelopmental, non-motor and sensory disorders influences the possibility of questioning the patient directly. Mental health can also be influenced by chronic pain, social isolation, functional deficiency and loss of autonomy. The patient’s intellectual level should therefore be explored and questionnaires adapted to the child’s age should be used.

Depending on the context, the HRQOL can be used:

- to describe the HRQOL of different groups (for example: groups of children with motor deficiencies; groups of children with no known health problems; HRQOL of children with different grades of motor deficiencies);
- to predict future progress for prognostic purposes: good results in the social dimension of the HRQOL in a child with a motor deficiency can be a predictive factor of good treatment-induced functional outcome in adolescence or adulthood;
- to evaluate changes that occur over time (natural progression or associated with treatment). The impact of different surgical strategies on the HRQOL can be evaluated in clinical trials.

3.3. Examples of applications in pediatric orthopedics

The quality of pediatric orthopedic evaluations varies considerably depending on the specific problem being studied. Reading an evaluation filled with statistics and the technical language of the research method is still difficult for many, emphasizing the need for training in this domain.

To facilitate critical analysis of an evaluation or a review of the literature, reading guides on the quality of the outcome evaluation have been proposed in the literature in other professional fields. Because the methodological bases are the same, these can easily be adapted to our medical field [29]. Thus, based on the reading guide and the ICF, we can form an opinion on the quality of the outcome evaluation (Box 3).

New approaches to the evaluation of treatment outcome can be found in the literature with measurement tools based on ICF principles, for example, the evaluation of clubfoot using the “clubfoot assessment protocol”, a global assessment tool (CAP) [30].

To perform a systematic analysis of study methodologies, they can be classified by ICF domain and/or by the EBM for the type of study and to determine the level of evidence of the reported results (Fig. 1).

Let us take the example of a study in the literature on the outcome of treatment of congenital spine deformities, a field that has progressed significantly in the past ten years.

By associating two keywords “congenital spine” and “outcome”, 558 articles were identified. After a more in-depth analysis and after excluding articles that were not related to the topic and technical notes or simple case studies, 76 articles remain, published between 1981 and 2012.

The EBM level was generally low: 70 level IV articles (retrospective case studies), with only 5 level III studies (well performed case–control studies) and one level II review of the literature.

In the area of outcome evaluation, all studies except one, focused upon the “function/structure” domain using traditional tools: clinical examination, changes in radiographic angles, CT scan, MRI or myelography, descriptions of the surgical intervention, immobilization and postoperative complications. Four more recent studies included parameters to evaluate pulmonary function.

Box 3: Reading guide on the quality of an outcome evaluation.

1. What ICF domain is targeted?
2. What is the goal of the outcome evaluation study?
3. What type of research is being performed (experimental, non-experimental, meta-analysis),
   a. Randomization procedure (if applicable).
   b. Stability of the control/comparative groups outside the intervention.
4. Evaluation of the results not considered for excluded patients.
5. Is the control/comparison group comparable to the treatment group?
6. Do the analyses correctly evaluate the results of the sub-group?
7. Are the measurement tools appropriate?
8. How long is the follow-up and does it make sense in relation to the goals of the study?
9. Does the report discuss the implementation of a treatment program?
10. Are the statistical tests appropriate and correctly interpreted?
11. Do the conclusions respond to the research question and to the goals of the study?

There are very few studies investigating other levels of the ICF: four studies evaluated “activity” and one “participation”. Seven studies measured quality of life with specific measurement tools (SF-36, CHQ, SRS-22).

In fact, the results in the literature provide very little evidence to help choose the best treatment. We have very little data on a child’s actual condition in daily life. It is also impossible to provide the public authorities with information on the cost of treatment or how the environment of these children and future adults needs to be adapted.

A review of the literature on the evaluation of treatment outcome in congenital clubfoot using the keywords “clubfoot” and “outcome” resulted in 508 articles. Using the same inclusion and exclusion criteria as in the preceding example, 274 articles were included. Sixty-nine percent of these articles had a mediocre level of evidence (IV). On the other hand, there were 52 level III articles, 29 level II articles and 3 level I articles. These outcome evaluation studies were usually limited to ICF criteria in the domain of “structure and function” (77%). A certain number (12.9%) analyzed day-to-day activities and none evaluated the patient’s participation in daily life. On the other hand, in the past 5 years, articles have been published that assess aspects of the environment (n = 6), satisfaction (n = 7), cost (n = 2) and quality of life in relation to health (n = 7).

These examples clearly show that the analysis of treatment outcome must change in the future. Evaluation tools based on ICF domains exist and can be applied to different pediatric orthopedic diseases, after confirming their validity in French (Table 3). A selection of quality of life questionnaires has been published which includes their psychometric properties [12]. Scientific societies must create database for each pediatric orthopedic disease to use for evaluation, to develop standardized longitudinal follow-up protocols and to propose this type of evaluation to healthcare authorities.

4. Perspectives and conclusions

Outcome evaluation is going to become increasingly important in pediatric orthopedics in the future as shown by the approach of the French National Health Authority which first concentrated on
improving professional practices and today is focused upon optimizing the management of healthcare services and improving the quality and safety of care.

There are very few existing studies in this field. Surgeons must reconsider their evaluation of patients and adapt techniques to children.

Follow-up based on childhood disease registers is not yet common in France, as it is in certain Scandinavian countries. To show the services rendered to the patient, longitudinal follow-up with regular evaluations could be proposed with treatment programs based on functional improvement of the patient as well as long-term improvement in participation and integration in social life.

In the future, multidimensional outcome evaluations should be designed for the different chronic diseases to help guide the practitioner's therapeutic choices.

The different players must be prepared to use these evaluation methods:

- surgeons – by obtaining training in the basic principles of this type of approach and method and especially by developing relationships with specialists in this domain;
- families and children – by being involved in regular outcome evaluation programs, which can be standardized by regional health authorities or on a national scale;
- the use of medical files will be expanded for patient management [31,32]; by creating centralized follow-up with “case managers” or “clinical file managers” for outcome evaluation and treatment programs. One option involves using “observatory” type files, standardized by major disease, with longitudinal follow-up.

Various approaches to automatic extraction of individual data from clinical files have been shown to be effective. These automatic systems can be associated with, or even replace manual methods of file review [33]. Collaborations with colleagues who treat adults must be improved to transfer our know-how and receive outcome evaluations on the social integration, daily life and quality of life of patients once they become adults [2].

We must go beyond the logic of being “obsessed with curves”: a precise measurement is not the answer to everything. Measuring the change in an angle following surgical correction of a deformity is not enough. It is the daily life and the quality of life following this correction that makes the difference. But these scales must not be used to avoid an intervention either, because longitudinal follow-up is indispensable. These new tools are not the answer to everything and must be used as a decisional aid while taking into account other factors as well.

New technologies for outcome evaluation should be searched for. Measurement of activities in real life situations with the help of a system of virtual reality will certainly be a technology of the future and an interesting field of research for future generations of pediatric orthopedists.

Outcome evaluation is a method that must be understood to improve the quality of care and service to patients. In the future, a global approach can be taken, including the medical, socioeconomic and managerial aspects to the problem. To optimize the information provided to the patients, this evaluation is indispensable, with two main goals:

- to perform this type of evaluation in daily practice in reference centers. However, we must be realistic, because medical teams specialized must first be trained in these methodologies and a clinical file must be created that can be used for longitudinal follow-up studies. All one needs to do is to look at one’s own healthcare facility to realize that this is a huge task;
- at the same time, simple evaluation protocols must be developed immediately based on quality of life questionnaires and functional scores using tests that are acceptable to all, because they do not yet exist.

These goals cannot be achieved without the support of scientific societies.

Disclosure of interest

The authors declare that they have no conflicts of interest concerning this article.

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