Functional and Symptom Impacts of Pediatric Head and Neck Lymphatic Malformations: Developing a Patient-Derived Instrument

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Otolaryngology -- Head and Neck Surgery 2012 147: 925 originally published online 6 June 2012
DOI: 10.1177/0194599812450838

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What is This?
Functional and Symptom Impacts of Pediatric Head and Neck Lymphatic Malformations: Developing a Patient-Derived Instrument

Karthik Balakrishnan, MD, MPH1, Todd C. Edwards, PhD2, and Jonathan A. Perkins, DO3

Sponsorships or competing interests that may be relevant to content are disclosed at the end of this article.

Abstract

Objective. Lymphatic malformations cause significant symptoms and functional deficits. Patients seek care for functional and symptomatic effects of their disease, but current disease burden and treatment outcome measures focus primarily on anatomy and histopathology. The authors describe disease impacts reported by patients and parents as a step toward more comprehensive disease burden assessments.

Study Design. Cross-sectional.

Setting. Children’s hospital vascular anomaly clinic.

Subjects and Methods. Participants were recruited through a pediatric vascular anomaly clinic. A panel of senior pediatric otolaryngologists and an outcomes scientist developed interview questions based on clinical and research experience and available literature. The outcomes scientist conducted parent and adolescent interviews. The panel reviewed responses to define relevant items within functional domains. Participants rated impact on daily life for each domain.

Results. Thirty-one participants represented all 5 de Serres stages (mean [SD] age, 9 [6] years; n = 11 adolescents and 20 parents). Adolescents reported frequent sickness as the domain with greatest impact. Sleep was more affected in adolescents with higher stage lesions. Parents of younger children reported greatest impact on breastfeeding. For adolescents, lesion stage predicted perceived social stigma (controlling for age), whereas increasing age was associated with greater impact from swelling (controlling for stage). For parents, stage predicted breastfeeding impact (controlling for stage).

Conclusion. This is the first detailed assessment of patient- and parent-reported functional and symptomatic impacts of head and neck lymphatic malformations. Both adolescent patients and parents of younger children reported significant symptom and functional effects of this disease.

Keywords
lymphatic malformation, vascular anomaly, pediatric otolaryngology, disease burden, patient centered

Received January 19, 2012; revised April 18, 2012; accepted May 16, 2012.

Lymphatic malformations (LM) are benign congenital malformations of lymphatic vessels. Their pathogenesis is not fully understood, although research in this area is progressing.1 They may occur within a broader syndrome2 or in isolation. Most LM are present at birth; the vast majority are clinically detected by age 2 years.3 Most do not regress spontaneously.4 Lymphatic malformations most commonly present in the head and neck,5 involving sites including the neck, face, tongue, mouth, and pharynx.

As might be expected given these head and neck sites, clinical experience suggests that LM may be associated with important symptom and function concerns such as difficulty with feeding or breathing sufficiently severe to warrant operative intervention.6,7 Patients may also experience bothersome pain and bleeding.7 Lymphatic malformations have been shown to be associated with lymphopenia,8 which has a functional impact via increased hospitalization and infection rates.9 Not least among the potential impacts of these lesions is perceived social stigma10 and associated problems such as speech and communication difficulty.
which clinical experience suggests are important parts of head and neck LM disease burden. These experiential problems drive head and neck LM patients and their parents to seek care and direct their treatment. However, no current measures of LM disease burden incorporate these impacts or allow measurement of treatment effects on disease burden. Rather, current descriptions of LM focus on simple anatomic and histopathologic lesion descriptions. These systems include the International Society for the Study of Vascular Anomalies classification, which places lymphatic malformations under the umbrella of low-flow vascular malformations and subdivides them into microcystic and macrocystic lesions. Even this important system, possibly the most comprehensive to date, is based on histologic and anatomic data without incorporating any subjective data. Although some classification systems are associated with treatment outcomes, outcome measures focus on clinicians’ assessment of lesion change or resolution or on treatment complications. At least one staging system, the Cologne Disease Score, is based on nonanatomic variables reflecting appearance, speech, swallow, and breathing, but that scale is based on observer ratings rather than on the patient’s own perceptions. Accordingly, it is reasonable to state that no adequate measure exists of the direct functional impact of LM on patients’ daily lives.

This study used face-to-face interviews to describe head and neck LM impact as perceived by pediatric and adolescent patients and their parents. It is intended as a first step toward a more comprehensive assessment of LM disease burden. This assessment in turn may be useful in treatment decisions and in developing outcome measures for LM treatment that incorporate the priorities of patients, parents, and providers.

Methods

Patients and Recruitment

Patients were recruited in 2009 and 2010 for this study through a pediatric hospital vascular anomalies clinic in Seattle, Washington. Inclusion criteria included diagnosis of head and neck lymphatic malformation, willingness to participate, and adequate cognitive/communicative ability on the part of parents and adolescent patients to provide informed consent. Exclusion criteria included unwillingness/inability of the patient or parent to participate. Approval was obtained from the Seattle Children’s Hospital Institutional Review Board.

Interviews and Data Collection

All interviews were conducted between April 2009 and April 2010. Using a grounded theory approach, interview questions were drafted by a senior pediatric otolaryngologist (J.A.P.) and an outcomes scientist (T.C.E.), with face validity based on clinical experience with head and neck LM, as well as on research experience in developing health interview questions. Criterion validity testing was not evaluated because of the lack of related “gold-standard” measures of LM disease impact. Questions were reviewed by a panel of pediatric otolaryngologists. These questions are presented as an appendix (available at otojournal.org) to this article. Semistructured interviews were conducted by telephone or in person by one author (T.C.E.), who has expertise in this method in both nonclinical and clinical populations, including adolescents with craniofacial anomalies. These methods have been described in detail by that author and the Seattle Quality of Life Group. For patients younger than 11 years, parents were interviewed. Patients 11 years and older were interviewed directly. Demographic and clinical data were collected on each patient, with LM stage recorded as previously described. Test-retest reliability and responsiveness to change were not evaluated because the study design did not include repeated testing of individuals or longitudinal evaluation.

Audio recordings of interviews were transcribed by a professional transcription service. De-identified full-text responses were reviewed by the authors (J.A.P. and T.C.E.) and coded for function- or symptom-related domains, with coding reconciled between reviewers. Interviewees were asked to rate the impact of head and neck LM on, or difficulty due to LM with, each domain using a 1 to 5 Likert scale (1 = not at all, 2 = a little, 3 = somewhat, 4 = quite a bit, 5 = a lot of impact). All domains were addressed with all interviewees, so there were no missing data. Impact ratings were obtained at the end of each qualitative interview, allowing the interviewer to ensure that unrated domains were considered “not applicable.” Thus, a “1” rating was considered applicable but no impact, whereas no rating was considered “not applicable.” As further participants were interviewed, questions were refined and new questions added based on earlier responses. Responses were presented to the panel of pediatric otolaryngologists, who extracted or highlighted key phrases or indicators of subjective symptom and functional impact. These extracted responses were used to provide further content validity in refining interview items.

Data Analysis

Data were entered in MS Excel (Microsoft Corp, Redmond, Washington). Analysis was performed using Stata/SE 9 (StataCorp, College Station, Texas). Impact ratings were recoded to a 0 to 4 scale, with 0 representing no impact and 4 representing the highest degree of impact. A mean rating of 1.0 or higher was chosen a priori to indicate a nonzero impact of LM for each domain. Based on the interview structure described above, missing values for each domain were treated as “not applicable” and were not included in mean score calculations. Multivariate analysis used linear regression.

Results

Thirty-five patients were recruited to participate in concept elicitation interviews and to complete symptom and function impact ratings. Eleven adolescent patients and 20 parents provided impact data and were included in the analysis, with patient age range from 21 days to 18 years (mean
Impact was rated on a scale from 0 through 4, with 0 indicating no effect and 4 indicating “a lot” of effect. Range of response and number of subjects for whom each domain was applicable are also presented. NA, not applicable.

Table 1. Patient and Parent Ratings of Impact of Head and Neck Lymphatic Malformations on Specific Function and Symptom Domains

<table>
<thead>
<tr>
<th>Impact Domain</th>
<th>Impact Rating by Adolescents, Mean ± SD; Range (No. of Respondents)</th>
<th>Impact Rating by Parents, Mean ± SD; Range (No. of Respondents)</th>
<th>Comparison, P Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain</td>
<td>2.30 ± 0.67; 1-3 (10)</td>
<td>3.00 ± 1.89; 1-4 (11)</td>
<td>.282</td>
</tr>
<tr>
<td>Bleeding</td>
<td>1.50 ± 1.38; 0-3 (6)</td>
<td>1.38 ± 1.06; 0-3 (8)</td>
<td>.857</td>
</tr>
<tr>
<td>Swelling</td>
<td>2.67 ± 0.52; 2-3 (6)</td>
<td>3.50 ± 0.58; 3-4 (4)</td>
<td>.046^</td>
</tr>
<tr>
<td>Perceived social stigma</td>
<td>2.67 ± 1.37; 0-4 (6)</td>
<td>3.57 ± 0.77; 2-4 (7)</td>
<td>.164</td>
</tr>
<tr>
<td>Chewing</td>
<td>1.50 ± 0.84; 1-3 (6)</td>
<td>2.60 ± 1.35; 1-4 (10)</td>
<td>.959</td>
</tr>
<tr>
<td>Speech</td>
<td>1.63 ± 1.30; 0-4 (8)</td>
<td>2.75 ± 1.28; 1-4 (8)</td>
<td>.104</td>
</tr>
<tr>
<td>Ability to turn head</td>
<td>3.00 ± NA; 3-3 (1)</td>
<td>2.50 ± 1.20; 1-4 (8)</td>
<td>NA</td>
</tr>
<tr>
<td>Need for special positioning</td>
<td>1.33 ± 0.58; 1-2 (3)</td>
<td>3.50 ± 1.00; 2-4 (4)</td>
<td>.021^</td>
</tr>
<tr>
<td>Sleeping</td>
<td>2.25 ± 1.50; 1-4 (4)</td>
<td>3.17 ± 1.17; 1-4 (6)</td>
<td>.306</td>
</tr>
<tr>
<td>Snoring</td>
<td>2.75 ± 0.71; 2-3 (2)</td>
<td>(0)</td>
<td>NA</td>
</tr>
<tr>
<td>Frequent sickness or illness</td>
<td>2.75 ± 0.96; 2-4 (4)</td>
<td>1.71 ± 1.11; 0-3 (7)</td>
<td>.152</td>
</tr>
<tr>
<td>Prolonged sickness or illness</td>
<td>2.50 ± 0.58; 2-3 (4)</td>
<td>4.00 ± 0.00; 4-4 (3)</td>
<td>.007^</td>
</tr>
<tr>
<td>Swallowing</td>
<td>2.33 ± 2.08; 0-4 (3)</td>
<td>3.14 ± 1.57; 0-4 (7)</td>
<td>.512</td>
</tr>
<tr>
<td>Breathing</td>
<td>2.60 ± 1.34; 1-4 (5)</td>
<td>4.00 ± 1.41; 1-4 (5)</td>
<td>.146</td>
</tr>
<tr>
<td>Apnea</td>
<td>(0)</td>
<td>2.00 ± 1.41; 1-4 (4)</td>
<td>NA</td>
</tr>
<tr>
<td>Stridor</td>
<td>2.00 ± 1.41; 1-3 (2)</td>
<td>1.00 ± 0.00; 1-1 (2)</td>
<td>.422</td>
</tr>
<tr>
<td>Restriction of usual activities</td>
<td>2.00 ± 1.53; 0-4 (7)</td>
<td>2.40 ± 1.67; 0-4 (5)</td>
<td>.676</td>
</tr>
<tr>
<td>Need for dietary modifications</td>
<td>1.80 ± 0.84; 1-3 (5)</td>
<td>1.57 ± 1.62; 0-4 (7)</td>
<td>.779</td>
</tr>
<tr>
<td>Oral care</td>
<td>1.00 ± 0.00; 1-1 (2)</td>
<td>1.33 ± 1.15; 0-2 (3)</td>
<td>.726</td>
</tr>
<tr>
<td>Normal development</td>
<td>(0)</td>
<td>(0)</td>
<td>NA</td>
</tr>
<tr>
<td>Breastfeeding</td>
<td>NA</td>
<td>3.78 ± 0.44; 3-4 (9)</td>
<td>NA</td>
</tr>
</tbody>
</table>

Impact ratings of 2.0 or greater: pain, swelling, perceived social stigma, sleep, snoring, prolonged sickness, swallowing, breathing, stridor, and activity restriction.

To further clarify the impact of LM on these domains, we stratified patients by de Serres stage, comparing stages 1-3 to stages 4-5 (Table 2). Direct comparison was not performed for several domains as they had zero or single patients reporting impact ratings, preventing calculation of a standard deviation to allow statistical testing. Of domains with ≥1 rating in each stage group, only sleep impact was significantly worse with higher-stage lesions.

Meanwhile, parents of younger children reported breastfeeding as most affected by head and neck LM (mean [SD] impact rating, 3.78 [0.44]). Other areas with parent-reported mean impact ≥2.0 were pain, swelling, perceived social stigma, sleep, snoring, prolonged sickness, swallowing, breathing, stridor, and activity restriction.

Exploratory multivariable regression analysis controlling for age and LM stage was used to examine the effect of these 2 variables on reported impact for those domains with mean ratings of 2.5 or greater. When controlling for age

[SD], 9 [6] years). All 5 de Serres LM stages were included (stage 1, 3 patients; stage 2, 13; stage 3, 4; stage 4, 3; and stage 5, 8). Lesions of several head and neck sites were represented in the sample, including face, lip, mouth, tongue, neck, larynx, airway, skull base, parotid, and ear canal.

A set of 20 domains was developed covering the variety of symptom and function impacts reported by adolescent patients and parents of younger children. These domains were pain, bleeding, swelling, perceived social stigma, chewing, speech, ability to turn head, need for special positioning, sleeping, snoring, frequent sickness or illness, prolonged sickness or illness, swelling, breathing, apnea, stridor, restriction of usual activities, need for dietary modifications, oral care, and normal development. A breastfeeding domain was included for parents but not for adolescents. Recoded impact ratings ranged from 0 (no impact at all) to 4 (a lot of impact). Nearly all domains showed a mean impact of 1 or greater for both adolescents and parents, meaning that LM had some impact on that domain of daily life (Table 1). For adolescents, only apnea and normal development were not rated as applicable by any subjects. Similarly, for parents, only snoring and normal development went unrated.

Adolescent patients rated frequent sickness (mean [SD] impact, 2.75 [0.96]) as the domain of greatest impact. Only 1 individual found head-turning restriction to be applicable and rated that impact as 3. Several other areas had mean impact ratings of 2.0 or greater: pain, swelling, perceived social stigma, sleep, snoring, prolonged sickness, swallowing, breathing, stridor, and activity restriction.

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among adolescent participants, LM stage was significantly associated with impact only for social stigma ($P = .037$). When controlling for LM stage, age was significantly associated with swelling impact ($P = .007$). When controlling for patient age in responses from parents, LM stage was significantly associated with impact on breastfeeding ($P = .005$). Age was not significantly associated with any impact rating when controlling for stage.

**Discussion**

Head and neck LM are relatively common, presenting in 1 of every 2000 to 4000 live births. These lesions may cause significant impairment, requiring extensive hospitalizations and frequent invasive interventions. In more severely affected patients, invasive therapies may show little benefit based on existing outcome measures and may lead to significant complications. Accordingly, providers must consider carefully the goals of any interventions. These goals may differ between patients, their parents, and providers.

Despite these issues, few studies address function and symptom impacts of LM on patients, appropriate management decision pathways, or appropriate outcome measures. Previous authors have examined general psychosocial effects of other pediatric vascular malformations on parents, overall quality of life of adult patients with LM and associated syndromes, and small sets of nonanatomic variables. However, no previous study has examined in detail the perspectives and priorities of patients and their parents in structured form.

This study is a first step in remedying that gap. As such, it has several strengths. No prior study has examined specific areas or domains of daily function in LM patients. It involved an experienced outcomes scientist in developing and administering interview questions and in analyzing the concept elicitation data. Questions were refined by a panel of pediatric otolaryngologists interested in head and neck LM. Adolescent patients and parents of younger children were examined separately, allowing consideration of the perceptions and priorities of each developmental group.

The study also has significant limitations. It was designed as a descriptive study and thus was not hypothesis driven. Multivariable analysis was purely exploratory to aid in future hypothesis generation. Sample size was also limited. A post hoc power analysis was performed as follows: 0.5 was chosen as the minimum effect size we would wish to detect. As the true standard deviation of our impact domains is unknown, we estimated impact rating standard deviation as 1.0 based on the standard deviations observed. With standardized effect size $0.5/1.0 = 0.5$, at least 88 patients are

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**Table 2. Patient Ratings of Impact of Head and Neck Lymphatic Malformations on Specific Function and Symptom Domains, Stratified by de Serres Lymphatic Malformation Stage**

<table>
<thead>
<tr>
<th>Impact Domain</th>
<th>Subjects with Stage 1-3 Lesions, Mean ± SD; Range (No. of Respondents)</th>
<th>Subjects with Stage 4-5 Lesions, Mean ± SD; Range (No. of Respondents)</th>
<th>Comparison, $P$ Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain</td>
<td>2.00 ± 0.63; 1-3 (6)</td>
<td>2.75 ± 0.50; 2-3 (4)</td>
<td></td>
</tr>
<tr>
<td>Bleeding</td>
<td>1.50 ± 2.12; 0-3 (2)</td>
<td>1.50 ± 1.29; 0-3 (4)</td>
<td></td>
</tr>
<tr>
<td>Swelling</td>
<td>2.67 ± 0.58; 2-3 (3)</td>
<td>2.67 ± 0.58; 2-3 (3)</td>
<td></td>
</tr>
<tr>
<td>Perceived social stigma</td>
<td>2.00 ± 1.73; 0-3 (3)</td>
<td>3.33 ± 0.58; 3-4 (3)</td>
<td></td>
</tr>
<tr>
<td>Chewing</td>
<td>1.00 ± 0.00; 1-1 (3)</td>
<td>2.00 ± 1.00; 1-3 (3)</td>
<td></td>
</tr>
<tr>
<td>Speech</td>
<td>1.50 ± 1.00; 1-3 (4)</td>
<td>1.75 ± 1.71; 0-4 (4)</td>
<td></td>
</tr>
<tr>
<td>Ability to turn head</td>
<td>3.00 ± NA; 3-3 (1)</td>
<td>(0)</td>
<td></td>
</tr>
<tr>
<td>Need for special positioning</td>
<td>1.00 ± 0.00; 1-1 (2)</td>
<td>2.00 ± 0.00; 2-2 (1)</td>
<td></td>
</tr>
<tr>
<td>Sleeping</td>
<td>1.00 ± 0.00; 1-1 (2)</td>
<td>3.50 ± 0.71; 3-4 (2)</td>
<td>.038&lt;sup&gt;a&lt;/sup&gt;</td>
</tr>
<tr>
<td>Snoring</td>
<td>3.00 ± NA; 3-3 (1)</td>
<td>2.00 ± NA; 2-2 (1)</td>
<td></td>
</tr>
<tr>
<td>Frequent sickness or illness</td>
<td>2.00 ± NA; 2-2 (1)</td>
<td>3.00 ± 1.00; 2-4 (3)</td>
<td></td>
</tr>
<tr>
<td>Prolonged sickness or illness</td>
<td>2.00 ± NA; 2-2 (1)</td>
<td>2.67 ± 0.58; 2-3 (3)</td>
<td></td>
</tr>
<tr>
<td>Swallowing</td>
<td>0.00 ± NA; 0-0 (1)</td>
<td>3.50 ± 0.71; 3-4 (2)</td>
<td></td>
</tr>
<tr>
<td>Breathing</td>
<td>2.67 ± 1.15; 2-4 (3)</td>
<td>2.50 ± 2.12; 1-4 (2)</td>
<td></td>
</tr>
<tr>
<td>Apnea</td>
<td>(0)</td>
<td>(0)</td>
<td></td>
</tr>
<tr>
<td>Stridor</td>
<td>3.00 ± NA; 3-3 (1)</td>
<td>1.00 ± NA; 1-1 (1)</td>
<td></td>
</tr>
<tr>
<td>Restriction of usual activities</td>
<td>1.25 ± 0.96; 0-2 (4)</td>
<td>3.00 ± 1.73; 1-4 (3)</td>
<td></td>
</tr>
<tr>
<td>Need for dietary modifications</td>
<td>2.00 ± 1.41; 1-3 (2)</td>
<td>1.67 ± 0.58; 1-2 (3)</td>
<td></td>
</tr>
<tr>
<td>Oral care</td>
<td>(0)</td>
<td>1.00 ± 0.00; 1-1 (2)</td>
<td></td>
</tr>
<tr>
<td>Normal development</td>
<td>(0)</td>
<td>(0)</td>
<td></td>
</tr>
</tbody>
</table>

Impact was rated on a scale from 0 through 4, with 0 indicating no effect and 4 indicating “a lot” of effect. Range of response and number of subjects for whom each domain was applicable are also presented. NA, not applicable.  
<sup>a</sup>Indicates a significant difference.
necessary to estimate LM impact to a granularity of 0.5. This sample size would be difficult to achieve because of the labor- and time-intensive nature of our study design, but future studies of our impact domains may help.

The study sample was limited to a single clinical site. Participants were recruited at a tertiary pediatric hospital, so the sample may underrepresent less clinically obvious or asymptomatic lesions. This in turn might lead to higher mean impact ratings in the study. Alternatively, our sample’s predominance of less extensive lesions (20/31 patients with de Serres stage 1-3 lesions) might be associated with overall lower mean impact ratings. This interpretation may be supported by finding that adolescents with stage 4 to 5 lesions had generally higher mean impact ratings for most domains, although statistical testing was largely precluded by our small sample size. Larger studies examining this question may help in incorporating objective disease extent into a composite staging system.

Finally, the study would be more robust if interview questions and impact domains had been developed in one sample and validated in a second sample, rather than relying on the concept elicitation sample entirely. However, the study’s focus was on the process of instrument development in LM patients and on identifying potential areas of future study. Given the time- and resource-intensive interview method, the goal was not to immediately describe LM impact on a population level. We also feel that similar methods might be useful in assessment of disease burden in other complex conditions.

These limitations aside, our data raise interesting points. First, adolescent LM patients and parents of younger LM patients did not agree perfectly on which domains showed greatest impact from head and neck LM. Although extensive statistical testing was prevented by sample size, parents appeared to rate impact on most domains noticeably higher than did adolescents. Such differences are not surprising, as perceptions of which domains are important in daily life would likely differ by age in any case. In turn, none of the high-impact domains identified by either group has been used previously by clinicians to assess disease burden, make treatment decisions, or determine treatment outcomes. Patients, parents, and providers may in fact have different goals for LM assessment and treatment. For example, very young children may not experience the same psychosocial impacts of LM as do adolescent patients. Such disparities exist in other chronic conditions, including musculoskeletal disorders and diabetes. Incorporating these differences into clinical decisions may produce improved subjective treatment outcomes and greater patient satisfaction. Understanding and describing these differences may also facilitate communication between various individuals involved in LM care, allowing providers to address the needs of all parties more effectively.

Second, both adolescents and parents identified pain as an important area affected by LM, although conceptual and methodological challenges affect proxy (parent) reports of pain. Although providers may assess LM patients’ pain, this variable may not factor sufficiently into treatment decisions, both acutely and for chronic pain control. We suggest that pain, and particularly oral pain, plays a major role in long-term outcomes of pediatric head and neck LM patients and warrants more attention than it currently receives. If oral pain is well controlled, patients may take better oral nutrition and maintain better dental hygiene. These activities in turn promote improved immune function and improved healing.

Third, both adolescents and parents rated LM impact as relatively high for social stigma, swelling, and frequent or prolonged sickness. These areas of daily function may be difficult for clinicians to address fully, particularly if they affect school attendance/performance or other “nonmedical” areas. The use of a multidisciplinary team has been advocated in complex conditions such as cleft lip and palate and pediatric hearing loss. Our data suggest that such a team might also benefit pediatric head and neck LM patients. Teams might include otolaryngologists, pediatricians, speech pathologists, nutritionists, psychologists, and social workers, for example. Interestingly, other authors have found that members of health care teams may not agree about goals of care. The use of detailed experiential data to describe disease burden, as in this study, may help team members communicate treatment goals, which in turn may improve overall care delivery. For example, breastfeeding impact was rated highly by parents, and counseling of parents by a lactation consultant and nutritionist early in an LM patient’s life may be beneficial.

Fourth, mean impact ratings varied widely between domains, suggesting that a composite measure of overall LM impact and disease burden may be useful in addition to assessment of individual domains. A patient’s experience comprises all of the studied domains and more, and individual domains cannot measure this experience in total and may underrate the overall effect of the disease on a child’s life. This emphasizes the need to develop more comprehensive measures of global disease burden, for which our data may prove useful.

Domain-specific data may be useful in suggesting further specific areas of questioning when providers take histories from LM patients and parents, as well as in future assessments of disease burden and treatment response. For example, the utility of prophylactic antibiotics in lymphopenic LM patients might be reflected in patient ratings of prolonged or frequent illness. Similarly, operative tongue reduction outcomes could be measured using impact scores for breathing, chewing, speech, and swallowing.

This study suggests that much further investigation is warranted into the perceived functional and symptom impacts of pediatric head and neck LM. Our data are preliminary, but they suggest a rationale for validated patient-derived disease burden measures. Supplementing current and future anatomic and histopathologic staging systems, such measures might be useful to clinicians, patients, and parents making decisions about the management of this complex disease.
Conclusion

This study provides the first detailed, patient-centered examination of head and neck LM impact. Our preliminary results suggest that adolescent patients place priority on different impact domains than do parents of LM patients. Both patient age and lesion stage may independently predict impact on some domains of daily life. Current disease burden measures, treatment decision pathways, and outcome measures do not consider such effects, although these problems lead patients and parents to seek medical care. Further study is needed to elucidate the utility of this study’s instrument and impact domains in assessing LM patient experience and disease burden.

Acknowledgments

We thank Fredric Hoffer, MD, for help conceptualizing this study and recruiting subjects and Stacy Russ for assistance in interview scheduling and manuscript preparation. We thank Donald L. Patrick, PhD, MSPH for consultation on instrument development. Scott Manning, MD, Andrew Inglis Jr, MD, Kathleen Sie, MD, Henry Ou, MD, and David Hom, MD, kindly served as pediatric otolaryngology panelists. All authors had full access to study data and take responsibility for data integrity and accuracy of data analysis.

Author Contributions

Karthik Balakrishnan, analysis, interpretation, article drafting and revision, final approval; Todd C. Edwards, conception, design, data acquisition, article revision, final approval; Jonathan A. Perkins, conception, design, data acquisition, article revision, final approval.

Disclosures

Competing interests: None.

Sponsorships: None.

Funding source: Seattle Children’s Hospital Academic Enrichment Fund 2008, #24833.

Supplemental Material

Additional supporting information may be found at http://oto.sagepub.com/content/by/supplemental-data

References


