A Staging System for Congenital Cholesteatoma

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Objective: To develop a staging system for congenital cholesteatoma in predicting the likelihood of residual disease.

Design: Retrospective analysis of data from a case series, to identify predictors of residual disease.

Setting: Tertiary care pediatric hospital.

Participants: Children undergoing surgical removal of congenital cholesteatoma. There were 156 patients, with 160 cholesteatomas; 4 children had bilateral disease.

Interventions: Each case was scored as to quadrants of the middle ear involved, ossicular involvement, and mastoid extension.

Main Outcome Measure: Surgically confirmed residual disease at any time after the initial procedure.

Results: Four stages were defined as follows: stage I, disease confined to a single quadrant; stage II, cholesteatoma in multiple quadrants, but without ossicular involvement or mastoid extension; stage III, ossicular involvement without mastoid extension; and stage IV, mastoid disease. There was a strong association between stage and residual disease, ranging from a 13% risk in stage I to 67% in stage IV.

Conclusions: This simple staging system may be particularly useful in standardizing the reporting of congenital cholesteatoma and in adjusting for severity in evaluating outcomes. It also provides information that is useful in counseling parents.

The classic presentation of congenital cholesteatoma is as a pearl behind an intact tympanic membrane in the anterior-superior quadrant, but congenital cholesteatoma can present elsewhere in the middle ear, can erode ossicles, and may extend to the mastoid. The extent of disease greatly affects the likelihood that residual cholesteatoma will remain after surgery. If one is to make comparisons among practices or to evaluate one’s own experience, it is necessary to adjust for severity of disease. Analyzing the case mix is essential to evaluating outcomes.

To adjust for severity, it is necessary to evaluate a rather large series in a consistent fashion, but no one hospital is likely to have enough cases for this. Recent series typically report that congenital cholesteatomas account for 10% to 28% of all pediatric cholesteatomas. As such, they are uncommon enough that few institutions have accumulated experience with a large series. At the Sixth International Conference on Cholesteatoma and Ear Surgery, 8 series of cases were presented. The median number of cases per report was only 40, and there was no consistency among authors as to classification systems. Thus, comparisons among series are difficult, and it is impossible to aggregate all the cases in any meaningful way.

The solution is to adopt a standard classification system. The value of such a system for combining data from many centers and risk adjustment is obvious, but the system would have other value as well. A simple classification system would be an aid to counseling families, allowing one to explain the prospects of residual disease in a particular case.

Classification systems for congenital cholesteatoma have been proposed before. We had previously used a staging system similar but not identical to the one described herein. The previous system did not adequately differentiate between disease confined to a single quadrant and more extensive disease, however. Roger et
al proposed a 4-category system, but it was not exhaustive in that it did not accommodate all possible cases. Grundfast et al described a meticulous reporting system in which presence or absence of disease is noted for each of 12 sites, but no simple staging system was proposed, and few others could reconstruct their findings in such detail from existing records.

**METHODS**

In a series of chart reviews that extended over 19 years, 160 cases of congenital cholesteatoma were identified at The Children’s Hospital of Philadelphia, Philadelphia, Pa. Approval of the Institutional Review Board at the hospital was obtained for this retrospective review.

Cholesteatoma was considered to be congenital if the tympanic membrane was intact and there was no history of otologic surgery (including myringotomy), otorrhea, or perforation. To rule out previous surgery or perforation, the hospital’s surgical database, as well as office charts (including medical history) and operative notes, was reviewed. Some early authors considered any middle ear disease an exclusion criterion, but in the present study we used the criteria of Levenson et al, who do not exclude cases of otitis media. In addition to history and physical examination, the findings at surgery were recorded. Presence or absence of disease was noted for each quadrant of the tympanic membrane at the time of surgery. Quadrants were defined as follows: The long process of the malleus was taken as the vertical axis. The horizontal axis was a line perpendicular to the vertical axis, passing through the umbo. Osseous involvement was noted. Cholesteatoma may erode the ossicles or be so adherent to the ossicles that portions of the ossicular chain must be sacrificed to eradicate disease. Also, on occasion, removal of ossicles may be required for access to the disease. It is recognized that the decision to sacrifice ossicles may be subjective, but this decision does reflect disease severity. For this review, destruction of any part of the ossicular chain, whether due to disease or surgery, was classified as osseous involvement. This category does not include cases in which cholesteatoma was dissected from the ossicles without compromise of the ossicular chain. Finally, extension into the mastoid was recorded.

Findings at subsequent surgical procedures were recorded, both for planned second looks or reconstruction and for surgery required by reappearance of disease. Any surgically confirmed cholesteatoma behind the tympanic membrane was presumed to be residual disease, regardless of location or size. Some authors differentiate between residual and recurrent disease, but it is not possible to determine whether subsequent disease arose from remnants of the original cholesteatoma.

Certainly, extent of disease affects audiological findings, but we decided not to consider the preoperative audiogram as a staging criterion. The reason is that precise ear-specific thresholds cannot be consistently obtained from younger patients. Audiometric data from a series that included the present cases have been analyzed and presented separately. Several considerations guided development of the staging system:

1. It should be simple. Surgeons are unlikely to complete a complicated rating system at the time of surgery. Fener still will have recorded sufficient detail in their operative notes that fine details could be reconstructed.
2. The classification system should be exclusive: there should be no ambiguity as to which category to use. It should also be exhaustive: there should be no cases that cannot be assigned to a category.
3. Categories should have good predictive value.
4. It is often best to avoid a category that has few cases, unless membership in that category has special significance. The reason is that the probability of a particular outcome, such as residual disease, cannot be estimated precisely with a small sample size.

**RESULTS**

Eighty-nine percent of the patients in this series were younger than 9 years at the time of surgery (mean age, 4.9 years). There was no significant left-right difference; 4 patients had bilateral disease. Seventy-one percent of the patients were male. For this study, follow-up only at our institution was analyzed. Eighty-four percent of cases were followed up for at least 1 year, and the mean duration of follow-up was 2.7 years. There was a 35% overall rate of residual disease.

The staging system was developed largely on the basis of clinical experience, but was modified and refined based on the predictive value of various possible definitions. As a first step, the predictive value of each dichotomous measure was determined using a binomial-probability test for significance of the difference between proportions. For example, there was osseous involvement in 69 cases, which was associated with a 0.59 chance of residual disease (Table). In the other 91 cases, there was a 0.16 chance of residual disease. With these sample sizes, the difference between 0.59 and 0.16 is highly significant (z=5.64; P<.001). One variable, the number of quadrants, was not dichotomous. Therefore, dummy variables were created: more than 1, more than 2, and more than 3. This univariate analysis, which does not take into account the relationships among variables, showed that mastoid extension, osseous involvement, and disease in more than 1 quadrant were all risk factors for residual disease. As for the number of quadrants, the risk level did not increase greatly if the criterion was more than 2 or more than 3. For this reason, only the single-multiple dichotomy was used in further analysis. The presence of disease in any quadrant other than the anterior-superior quadrant was a predictor of residual cholesteatoma, but there was a confounding variable: extensive disease was much more common in these cases.

The next step was to explore combinations of predictors. The best predictor of good outcome (no residual disease) was disease confined to 1 quadrant of the middle ear, without osseous or mastoid involvement. This extent of disease was defined as stage I. A preliminary version of the classification system defined stage I as disease limited to the anterior-superior quadrant. In this series, however, there were 10 cases of single-quadrant disease elsewhere (9 posterior-superior, 1 posterior-inferior) without osseous involvement or mastoid extension. Combining these cases with the anterior-superior cases increased the predictive power, using P level as a criterion.

Mastoid extension carried the highest risk of residual disease. Consideration was given to the predictive value of other factors within this category, to determine whether mastoid extension should represent the
most serious stage, regardless of other findings. It was found that most of these cases—35 of 39—also had ossicular involvement. Although it was noted that only 1 of the remaining 4 cases had residual disease, the numbers were too small for meaningful prediction, nor did the number of involved middle ear quadrants predict which of the mastoid cases would have residual disease. Therefore, the highest stage, stage IV, included all cases with mastoid extension, regardless of other findings.

There remained the cases with ossicular involvement, more than 1 quadrant, or both, but without mastoid extension. Within this group, ossicular involvement was a strong predictor of severity, regardless of the number of quadrants, and stage III was defined accordingly. Stage II included all cases with multiple quadrants but without ossicular or mastoid involvement. Predictive value was not increased by subdividing stage II according to the number of quadrants.

In summary, the stages are defined as follows:

- **Stage I**, single quadrant: no ossicular involvement or mastoid extension.
- **Stage II**, multiple quadrants: no ossicular involvement or mastoid extension.
- **Stage III**, ossicular involvement: includes erosion of ossicles and surgical removal for eradication of disease; no mastoid extension.
- **Stage IV**, mastoid extension (regardless of findings elsewhere).

**Figure 1** shows the percentage of cases that fell within each of the 4 stages. Most cases were classified as stage I, with the remainder rather evenly distributed among stages II, III, and IV. In **Figure 2**, it can be seen that there is a clear association between stage and likelihood of residual disease, ranging from 13% at stage I to 67% at stage IV.

**Comment**

This straightforward classification system is easy to use and has good predictive value. We have found it to be quite simple to apply and will use it prospectively as well as for analysis of past cases. Its potential will not be fully realized, however, unless it is widely accepted and applied to the reporting of experience from many centers. Will this happen? It is possible that it will. The great majority of reports of experience with pediatric congenital cholesteatoma are made by otologists or pediatric otolaryngologists. A good start would require only the consensus of these 2 groups.
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REFERENCES