**Congenital Cholesteatoma**

**Predictors for Residual Disease and Hearing Outcomes**

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**Objective:** To determine predictive factors for residual disease and hearing outcomes of surgery for congenital cholesteatoma (CC).

**Design:** Retrospective record review of surgery for CC from January 1, 1998, through December 31, 2010. The initial extent of CC was staged using the system as defined by Potsic et al.

**Setting:** Tertiary care children’s hospital.

**Patients:** Eighty-one children (82 ears) underwent a total of 230 operations for CC. The mean (SD) age was 5.3 (2.9) years, and the mean follow-up was 4.3 years.

**Intervention:** Initial and subsequent operations for CC and audiologic evaluations.

**Main Outcome Measures:** Statistical analyses were performed to determine factors associated with increased residual disease for CC and poorer hearing outcomes.

**Results:** Higher initial stage of disease, erosion of ossicles, and removal of ossicles were significantly associated with increased likelihood of residual CC (46%, 50%, and 51%, respectively; \( P < .001 \)). More extensive disease at initial surgery was associated with poorer final hearing outcomes (\( P < .05 \)). Other significant findings included CC medial to the malleus (41.5%) or incus (54.3%), abutting the incus (51.3%) or stapes (63%), or enveloping the stapes (50%); all patients had increased residual disease (all \( P < .05 \)). Excellent audiometric results (air-bone gap of \( \leq 20 \) decibel hearing level) were obtained in 63 (77%) of the 82 ears.

**Conclusions:** More extensive initial disease, ossicular erosion, and the need for ossicular removal were associated with residual disease. On the basis of our data, the best chance for completely removing CC at initial surgery involves removing involved ossicles if they are eroded, if the CC is abutting or enveloping the incus or stapes, if the CC is medial to the malleus or incus, or if the matrix of the CC is violated. These results may help guide surgeons to achieve the best results for their patients.


**Congenital Cholesteatoma (CC) is a unique pathologic entity that has strict criteria distinguishing it from acquired cholesteatoma. The prevalence of CC is low (2%-5% of cholesteatomas). Thus, a single surgeon’s experience treating CC may be limited. In an attempt to maximize favorable treatment outcomes in children with CC, 2 staging systems have been proposed on the basis of 2 large series at tertiary care children’s hospitals.1,3**

Nelson et al2 characterized type 1 lesions as involving the middle ear without ossicular involvement. Type 2 lesions involve the ossicles, with ossicular involvement defined as ossicular erosion or removal of the ossicles due to disease. In the staging system by Potsic et al,1 CC is classified into 4 stages with single-quadrant disease as stage 1. Stage 2 disease involves multiple quadrants. Stage 3 is ossicular involvement, defined as erosion of ossicles and surgical removal for eradication of disease. Finally, stage 4 disease is any mastoid extension.3 We have observed at Children’s Hospital of Pittsburgh of the University of Pittsburgh Medical Center (UPMC) that involvement of the ossicles may take several different forms. Outcomes for CCs that could be peeled off intact ossicles without opening the matrix were often different from outcomes for CCs that abutted ossicles but had violation of the matrix. Disease that was medial to the malleus and incus and adherent to or wrapped around the stapes was often difficult to remove and may have resulted in high residual disease rates.
In light of these observations, we sought to systematically review our CC surgical experience during the past 12 years at Children’s Hospital of Pittsburgh of UPMC, evaluating the initial extent of the CC, the technique used for removal, and the outcomes (residual disease and hearing). We sought to identify predictive factors that may help to guide surgeons in their surgical planning, operative technique, and follow-up treatment. In doing so, we also hoped to supplement the already established staging systems to more completely classify CC into treatable stages that guide management decisions, including type of surgical intervention, intraoperative decision making, hearing outcomes, and the need for postoperative monitoring for possible residual CC that requires additional surgery.

METHODS

This was a retrospective medical record review from January 1, 1998, through December 31, 2010, at Children’s Hospital of Pittsburgh of UPMC. Approval of the institutional review board at the University of Pittsburgh Medical Center was obtained. All records of pediatric patients who were diagnosed as having cholesteatoma were initially reviewed. The patient was considered to have a CC if he or she met the following inclusion criteria: CC present, normal pars tensa and flaccida, no history of otosclerosis, no tympanic membrane perforation, and no previous otologic procedures. Patients were included in the study if they were scheduled for tympanosotomy tube placement and the CC was diagnosed intraoperatively. Also, patients with a history of otitis media were included per the criteria of Levenson et al. Once patients were identified who met the aforementioned criteria, their medical records and operative reports were reviewed and a database was established recording the following variables for all patients:

1. Demographic characteristics: age; sex; and CC right, left, or bilateral
2. History: history of otitis media, preoperative computed tomography scan, and surgeon performing surgery
3. Primary intraoperative findings: stage (using the Potsic et al criteria); location; quadrants involved; intact pearl with intact matrix vs more diffuse disease with matrix violated; location relative to ossicles; involvement of ossicles: abutting, enveloping, or eroding; involving tensor tympani tendon; CC present in attic, hypotympanum, and protympanum; facial recess; sinus tympani; and blockage of Eustachian tube
4. Procedures performed: primary surgery, removal of ossicles initially or at second or third look, dissection of CC off ossicles, number of subsequent operations, and type of ossicular reconstruction
5. Outcomes: preoperative hearing (reported as the pure-tone average [PTA] at 500, 1000, and 2000 Hz in decibel [dB] hearing level [HL]); postoperative PTA at ages 6 months, 1 through 5 years, and older than 5 years; years of follow-up; residual disease; location of residual disease; and need for tympanosotomy tubes

Selected important characteristics for each event are summarized in Table 1.

Residual disease was specifically defined as surgically and pathologically confirmed cholesteatoma in the following locations: hypotympanum, cochleariform process, involving each ossicle, stapes footplate, fallopian canal, oval window, medial surface of the tympanic membrane, Eustachian tube, and protympanum. External auditory canal and intratympanic memb
to 12 years). Fifty-six patients (69%) were male and 25 (31%) were female. There was no significant side predominance, with 54% (44 of 82) right-sided CC and 46% (38 of 82) left-sided CC. The mean (SD) age at diagnosis was 5.3 (2.9) years. The mean age based on stage increased from stage 1 to 3 (3.9 years [stage 1], 4.2 years [stage 2], 6.4 years [stage 3], and 6.2 years [stage 4]). Fifty patients (62%) had a history of acute otitis media before surgery.

Preoperative hearing data revealed a mean (SD) PTA of 20 (11) dB HL. When evaluated by stage, increasing stage correlated with increased preoperative hearing loss reported as PTA (P < .05) (Figure 1).

The initial extent of disease was categorized by stage using the Potsic et al criteria. The largest group within our cohort was stage 3, with 34 cases (41%). The smallest group was stage 4, with 11 cases (13%). Comparing with previous studies, the anterosuperior quadrant was the most common quadrant involved (70 of 82 or 85%). Of note, 65% of cases (53 of 82) also involved the posterosuperior quadrant. Single-quadrant cases accounted for 24% of cases (20 of 82). Most cases of single-quadrant disease were in children younger than 3 years.

The surgical approach was transcanal tympanoplasty, endaural tympanoplasty, or postauricular for all initial tympanoplasties. All initial tympanomastoidectomies were canal wall-up procedures. Eleven patients (14%) underwent a single procedure with no second look or residual disease observed on office follow-up. Eighty-seven percent (70 of 82) of cases underwent a second-, third-, or fourth-look surgery for residual disease or ossicular reconstruction. A total of 230 operations were performed for 82 CCs. Seventy children (86%) underwent a second-look; 21 (26%) underwent a third-look, and 17 (21%) underwent a fourth-look surgery for residual CC or ossicular reconstruction. Thirty-seven patients (46%) underwent ossicular reconstruction, and 1 (82%) is still pending reconstruction.

Middle-ear residual disease was encountered in 27 cases (33% of the cohort). Neither patient age, sex, nor right vs left ear were associated with the presence of residual disease (P = .09, P = .31, and P > .99, respectively). Residual disease rates increased significantly with increasing disease stage (P < .001). The rates of residual CC identified at subsequent operations by stage were 5% (stage 1), 24% (stage 2), 44% (stage 3), and 64% (stage 4).

The mean age of patients with residual disease was 5.8 years. Mean ages by stage for children with residual disease were 2.7 (stage 1), 5.4 (stage 2), 6.3 (stage 3), and 6.2 years (stage 4). One or more ossicles were removed in 45% of the cases (37 of 82), and residual disease was encountered in 51% of cases (42 of 82) where the ossicles were removed at initial surgery. Removal of 1 or more ossicles was significantly associated with presence of residual disease (P < .001).

Ossicular involvement was defined in a detailed fashion as described in the “Methods” section. Having an initial intact CC pearl that was not adherent to ossicles was statistically significantly associated with greatly reduced middle-ear residual disease rates at second-look surgery (P = .01). The following detailed characteristics of ossicular involvement were significantly associated with middle-ear residual disease: CC medial to the malleus or incus, CC abutting the incus or stapes, CC enveloping the stapes, stapes erosion, and ossicle(s) removed at initial surgery (Table 2). Congenital cholesteatoma involving the tensor tympani tendon tended to be associated with middle-ear residual disease, but this did not reach statistical significance (P = .06) (Table 2). The tensor tympani tendon was divided to assist in removal of the malleus to allow improved access to the anterior epitympanum and the mesotympanum. The tensor tympani tendon was not divided without removal of the malleus.

We compared the residual disease rates of CC that were medial to the malleus in which the surgeon dissected the CC off the ossicles and retained them vs removal of the ossicles entirely and found no statistical difference in residual disease rates between the 2 groups (P = .33). The patients who had ossicles removed at initial surgery had a higher residual disease rate, possibly due to more advanced initial disease. There is support for this concept because we determined that disease stage and removal of ossicles status were associated with each other (P < .001; Fisher exact test) and both were independently associated with the presence of residual disease (P = .001 and P < .001, respectively; Fisher exact tests), indicating the presence of confounding.

Of the 82 cases, there were 4 cases in which the malleus alone was removed. One of the 4 (25%) had residual CC. In 50 cases, the incus was retained in its entirety with a residual CC rate of 20% (10 of 50). There were no cases of only incus removal. There were 27 cases in which the incus and malleus were removed with a residual CC rate of 32% (14 of 27). There were 5 cases in which the malleus, incus, and part of the stapes (either posterior or anterior crura) were removed with a residual CC rate of 60% (3 of 5). There was a statistically significant difference between the 3 groups (P = .007; Fisher exact test), indicating that the need for removal of additional ossicles was associated with an increased...
rate of residual CC. We implemented multivariable logistic regression analysis to evaluate whether an increasing number of ossicles removed during the surgical procedure was associated with residual disease even after controlling for stage of disease. In a univariate logistic regression model, removal of the incus and malleus was associated with significantly increased residual disease compared with retention of the incus (odds ratio, 4.3; 95% CI, 1.5-12.0). Removal of the incus, malleus, and stapes was only performed in 5 CC cases, and this tended to be associated with increased residual disease (odds ratio, 6.0; 95% CI, 0.8-40.8). However, in multivariable logistic models adjusted for stage, an increasing number of ossicles removed during the procedure used was not significantly associated with residual disease (both \( P > .45 \)), indicating that the number of ossicles involved did not impact residual disease rates independent of disease stage.

Immediate (4-6 weeks) postoperative hearing results were available for 98% of patients (80 of 82) in the cohort. The mean (SD) postoperative PTA in the immediate period was 24.4 (13.7) dB HL, at 6 months was 23 (13) dB HL, and at 5 years was 23 (15) dB HL. Follow-up hearing results 5 or more years after surgery were available for 47 patients (58%). The average gain in hearing on the most recent audiogram following surgery by stage was 0 dB HL (stage 1), 2.6 dB HL (stage 2), 4.2 dB HL (stage 3), and 5 dB HL (stage 4). The most recent audiogram for each patient in the cohort yielded a mean PTA of 21 dB HL. Evaluated by initial stage, the most recent audiometric result averages were 14 dB (stage 1), 13.5 dB (stage 2), 25 dB (stage 3), and 35.5 dB (stage 4) (Figure 1). The range of most recent audiogram results was 6 months to 12 years after initial surgery. There was a statistically significant difference in most recent audiometric results between stage 1 and 2 vs stage 3 and 4 results (\( P < .05 \)). Excellent audiometric results with PTA air-bone gap of 20 dB HL or less were obtained in 63 (77%) of 82 ears.

Although the use of otoendoscopes and lasers has likely increased in more recent years, our study did not capture these data.

Complications associated with the 230 operations performed were rare. No facial nerve injuries, sensorineural hearing loss, cerebrospinal fluid leak, or wound breakdown were encountered. There was 1 meningocele, 1 hypermobile stapes footplate, and 1 external auditory canal stenosis. The canal stenosis was repaired surgically by drilling the external auditory canal and placing a split-thickness skin graft. The meningocele was repaired using a cartilage graft to repair the tegmen defect, and the hypermobile stapes footplate was observed with no surgical intervention necessary. Seventeen patients (21%) had recurrent otitis media after their surgery, and 20 (25%) required tympanostomy tubes at some point after their initial surgery.

**COMMENT**

Two CC staging systems have been devised with the goal of creating a common language for reporting cases and comparing similar extent of disease and outcomes. Our goal was to further refine those staging systems to determine factors significantly contributing to residual disease and to improve intraoperative decision making based on location of disease.

Our results validated the Potsic et al staging system for CC. As stage increased from 1 through 4 within our series, the rates of residual disease increased progres-
The number of operations each patient may require. There may be exceptions to the algorithm. For example, rupture of the matrix with no ossicular involvement would not warrant removal of ossicles.

When comparing our data with that by Nelson et al,2 we did have concordant rates of preoperative acute otitis media: 49% for the Nelson group and 61% in our study. In contrast, a series of 44 CCs studied by Karmarkar et al8 found isolated posterosuperior mesotympanum CC in 21 patients (48%), whereas most of our isolated single-quadrant disease cases (n = 17) were in the anterosuperior quadrant. Our similar findings were thus consistent with several other series.1,2,5,7,8

The natural history and progression of CC was described using intraoperative illustrations by Koltai et al7 in 2002. They described CC progression from the anterosuperior quadrant, to the posterosuperior quadrant (with ossicle destruction), to the attic, and then to the mastoid. They also discussed the possible end point of the natural history of CC to be a young child with otorrhea, maximum conductive hearing loss, tympanic membrane perforation in a nontraditional location, and a middle ear and mastoid full of cholesteatoma. Our ability to identify “end point” CC is limited because of the definition of CC diagnosis being based on an intact tympanic membrane. This is a limitation of our series and may bias our cohort toward less advanced disease.

Another limitation to our study is the large group of surgeons who participated in this series (n = 11) with years of experience ranging from 1 to 33 years. We did analyze surgical outcomes vs years of experience and found no significant differences. The retrospective nature of this study has intrinsic limitations. Although all data concerning CC were collected from each medical record, it is possible that the surgeons omitted some data on some cases in the operative reports in the absence of a prospective study.

There was an average of nearly 3 operations per CC. This is a relatively high rate of revision surgery for CC. The explanation for this high rate of revision surgery is related to the philosophy of planned avoidance of canal wall-down, open-cavity surgery in children at our institution. Our protocol has been to perform additional revision surgery when possible rather than perform canal wall-down surgery that may bring about life long open-cavity problems. Conversely, without taking down the canal wall, residual CC may be more frequent. Thus, the high rate of revision surgery may reflect the protocol at our institution and may not represent the practice at other institutions. Thus, the decision for canal wall-up vs canal wall-down surgery is left to the discretion of the surgeon. Although the otoendoscope and laser use was not captured in our study, an acknowledged limitation, we speculate that the use of otoendoscopes and lasers may further decrease the incidence of residual CC.

The role of radiographic imaging in the diagnosis, management, and follow-up of CC is evolving. El-Bitar et al9 published a series describing 35 patients with CC demonstrating that preoperative computed tomography scan accurately predicted the initial location and extent of CC that could not be accurately assessed on otomicroscopy. We agree that preoperative computed tomography is es-

![Figure 2. Surgical algorithm: If the matrix is intact, then the ossicles do not need to be removed. If the cholesteatoma is abutting or enveloping the incus, medial to the malleus and incus, enveloping the stapes, or eroding the ossicles, then the involved ossicle(s) should be removed.](https://example.com/image.png)
sential in defining the extent of existing pathologic features, and all our patients undergo preoperative computed tomography. More important, there are promising new data using diffusion-weighted magnetic resonance imaging to monitor patients for residual or recurrent cholesteatoma.10,11 As this technique is validated by more prospective trials in the future, it is likely to become a non-invasive method for monitoring at-risk patients with postsurgical stage 3 and 4 CC and to prevent the need for all such patients to be taken back to the operating room to address possible residual disease.

The postoperative hearing results in our series compare well with other series that report up to and greater than 5-year postoperative results. The most recent audiograms for the entire cohort revealed excellent audiometric results. Air-bone gap of 20 dB HL or less was obtained in 63 (77%) of 82 ears. Benhammou et al12 reported a 50% rate of this level of hearing after 5 years, and Doyle and Luxford13 reported that 91% of patients had postoperative PTA air-bone gap of 20 dB HL or less in their series of 60 cases.

In conclusion, CCs have been classified on the basis of their presence in quadrants of the middle ear and whether they erode or necessitate removal of the ossicles in past staging systems.1,2 Our results validated the staging system of Potsic et al3 in that higher initial stage of CC, erosion of ossicles, and removal of ossicles predicted significantly higher residual rates. Also, higher initial stage of CC predicted poorer final hearing outcomes. Important additional findings in the present study included the observation that CC that is medial to the malleus or incus, abutting the incus or stapes, or enveloping the stapes should be included in the Potsic stage 3 classification on the basis of our finding of significantly increased residual disease rates for these cases. On the basis of our data, the best chance for completely removing CC at initial surgery will include removing involved ossicles if they are eroded; if the CC is medial to the malleus or incus, abutting the incus or stapes, or enveloping the stapes; or if the matrix of the CC is violated. These recommendations may help guide surgeons to achieve the lowest residual rates for their patients with CC. Finally, the future use of diffusion-weighted magnetic resonance imaging may be valuable for detection of residual disease and thus decrease the need for universal second-look surgery in this group of high-risk patients.

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REFERENCES


